Quality of Life in Pediatric Patients with Intestinal Failure on Home Parenteral Nutrition

by

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A thesis submitted in conformity with the requirements
for the degree of Master of Science (Clinical Epidemiology)

Graduate Department of Health Policy, Management and Evaluation
University of Toronto

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Abstract

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Introduction: Medical advances have resulted in reduced mortality of pediatric patients with Intestinal Failure. Consequently, more patients go home on parenteral nutrition (PN) for extended durations. This time-consuming and complicated therapy necessitates persistent vigilance in monitoring and response to potential life-threatening side effects. These issues may impact quality of life (QOL) for patients, caregivers and families.

Methods: This observational, cross-sectional, mixed-methods analysis of multidimensional QOL used a quantitative battery assessment and a qualitative focus group. Questionnaire results were compared to published norms, published small bowel transplant (SBTx), and institutional SBTx patients.

Results: Home PN proxy assessments scored children lower than published norms but similar to SBTx. The child self-reports were similar to both normative and SBTx populations, except lower general and overall health. Generic questionnaires did not capture disease-specific issues.

Conclusion: QOL is compromised in children on home PN and caregivers compared to norms but is similar to SBTx.
Acknowledgements

I would like to thank my husband, Jonathan, for all of his patience and understanding throughout these past two years. I consider myself very lucky to have a wonderful family that has stood by me through many years of school and through all of my endeavors in life.

To my advisor, Dr. Paul Wales, for his guidance, support and direction. It was a great pleasure to work with someone who was always enthusiastic about this research, understanding about unexpected life events and extremely supportive at every step.

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*Note: Copies of additional scales from battery assessment are not included in appendices due to copyright issues
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<td>ADHD</td>
<td>Attention Deficit Hyperactivity Disorder</td>
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<td>BANS</td>
<td>British Artificial Nutrition Survey</td>
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<tr>
<td>BSI</td>
<td>Brief Symptom Inventory</td>
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<tr>
<td>CBCL</td>
<td>Child Behaviour Checklist</td>
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<td>CF</td>
<td>Cystic Fibrosis</td>
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<td>CHIP</td>
<td>Child Health Inventory for Parents</td>
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<td>CHQ</td>
<td>Child Health Questionnaire</td>
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<td>General Health Questionnaire</td>
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<td>GIFT</td>
<td>Group for Improvement of Intestinal Function and Treatment</td>
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<td>HRQOL</td>
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<td>Health Utilities Index</td>
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<tr>
<td>ID</td>
<td>Identification</td>
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<tr>
<td>IFALD</td>
<td>Intestinal Failure Associated Liver Disease</td>
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<td>ITQOL</td>
<td>Infant Toddler Quality of Life Questionnaire</td>
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<tr>
<td>JRA</td>
<td>Juvenile Rheumatoid Arthritis</td>
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<td>KS</td>
<td>Kolmogorov-Smirnov</td>
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<td>NICU</td>
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<td>OHIP</td>
<td>Ontario Health Insurance Program</td>
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<td>PedsQL</td>
<td>Pediatric Quality of Life Inventory</td>
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<td>Parenting Stress Inventory</td>
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<td>Quality of Life</td>
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<td>QOLI</td>
<td>Quality of Life Inventory</td>
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<td>SBS</td>
<td>Short Bowel Syndrome</td>
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<td>SBTx</td>
<td>Small Bowel Transplant</td>
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<td>Short Form (36) Health Survey</td>
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<td>SIP</td>
<td>Sickness Illness Profile</td>
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<td>TNO-AZL Preschool Children Quality of Life Questionnaire</td>
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<td>VAS</td>
<td>Visual Analogue Scale</td>
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Chapter 1
Introduction

Treatment of children with intestinal failure has advanced and resulted in reduced mortality. As a result, more children are going home on parenteral nutrition (PN) for extended periods of time. The caregivers of this new generation of children on long-term home PN and the children themselves are expected to maintain intensive schedules of nutritional supplementation via central venous catheter and to monitor for life threatening side effects. It is essential to understand the experience and quality of life (QOL) of the children and their families who are expected to utilize this complex therapy.

Small bowel transplantation (SBTx) is provided to those children who experience life-threatening morbidity on PN. Quality of life is not formally part of the transplant criterion although it may be considered when listing a child for transplant. As outcomes for SBTx improve, it has been speculated that poor QOL on home PN will be considered as criterion for transplant listing. At present, the long-term survival for home PN is significantly better than SBTx and thus listing is reserved for complicated cases of intestinal failure and morbidity that would result in death. However, it remains important to compare the QOL between these two populations to determine the degree to which QOL improves post transplant.

Therefore, this study was designed to describe the QOL in this new generation of home PN patients and to compare to published normative data as well as to published data of patients who had a SBTx. In addition, QOL was assessed in a small sample of SBTx patients at our own institution. The goal of the study was to describe the new population of home PN patients in terms of QOL, to describe the impact on the primary caregivers and the families of these children and to compare the QOL of patients on home PN versus post SBTx. It is our aim to provide optimal support for these families and children in order to facilitate coping and adaptation of treatment and implications of intestinal failure.
Chapter 2
Literature Review

2.1 Intestinal Failure

Intestinal failure occurs when there is reduced intestinal absorption requiring macronutrient, water and electrolyte supplementation to maintain health. Intestinal failure is considered to be a functional rather than an anatomical condition. It describes any condition in which enteral supplementation via the gastrointestinal tract fails to satisfy nutritional, electrolyte and fluid requirements to maintain health and/or growth. Parenteral nutrition (PN) is the primary therapy for intestinal failure providing supplemental intravenous nutrition via central venous catheter to sustain growth and development and to prevent nutritional deficiencies. There are multiple etiologies of pediatric intestinal failure including congenital or surgically acquired short bowel syndrome (e.g. gastroschisis, necrotizing enterocolitis, intestinal atresias), neuromuscular intestinal disorders (e.g. chronic intestinal pseudo-obstruction syndrome), or severe protracted diarrhea due to intestinal disease (e.g. microvillous inclusion disease). Intestinal failure in adults is often a result of much different causes than in pediatrics and tends to fall into three groups: 1. patients who were previously well and had suffered a catastrophic event e.g. volvulus, mesenteric thrombosis; 2. patients with Inflammatory Bowel Disease who were unresponsive to treatment; 3. patients with malignancy causing intestinal obstruction or intestinal failure due to radiology. An important difference between adult and pediatric intestinal failure is that most children are started on PN before the age of 1 year unlike adults who are often started on PN after experiencing life without this therapy. This results in a different frame of reference when assessing quality of life (QOL) on home PN.

2.1.1 INTESTINAL ADAPTATION

Intestinal adaptation involves physiological changes in the remaining intestine that result in improved overall absorptive capacity. Adaptation facilitates the achievement of the ultimate intestinal failure treatment goal: intestinal autonomy and PN independence. Children whose bowels adapt have improved absorption and will eventually have their PN discontinued. Factors thought to impact epithelial growth of the normal small bowel include genetic pre-programming, growth factors in breast milk, luminal factors (e.g. food, bile, pancreatic secretions), endocrine...
hormone effects, enteric/systemic nervous system effects and gut-associated lymphoid tissue mediated effects. In a retrospective review of intestinal failure patients at the Hospital for Sick Children in Toronto, approximately 64.8% of infants with short bowel syndrome achieved adaptation. Children with intestinal failure who do not demonstrate adaptation require partial or complete PN for an indeterminate period of time. Children with neuromuscular intestinal disorders and many diseases of severe protracted diarrhea are often unable to achieve adaptation, unable to be weaned from PN and may require a small bowel transplant (SBTx). Other surgical, non-transplant approaches to facilitate adaptation include surgeries to lengthen the bowel, improve intestinal function, improve motility or increase adaptive surface area. At present, although there are many potential predictors of adaptation, the ability of a medical team to predict a patient's success of achieving intestinal adaptation remains quite poor and thus determining the need to list for small bowel transplantation can be challenging.

### 2.2 Parenteral Nutrition

#### 2.2.1 HOME PARENTERAL NUTRITION

For children who are expected to be on PN for an extended period of time, the option of administering PN at home is offered to the family. The child is discharged with a central venous catheter, the PN and intravenous solutions to maintain their nutritional and fluid status as well as supplies and equipment to administer and monitor PN. The children may also require gastrostomy tube feedings, specialized enteral formulas and/or diet restrictions. The primary caregiver participates in an intensive education program and must be assessed as competent in PN administration and sterile technique prior to allowing the patient to be discharged home on PN. Although there is some available nursing support in the community, the family is expected to provide the vast majority of the care. Caregivers of children on home PN and the children themselves are required to provide complex care and must maintain difficult schedules while monitoring and responding to potential life threatening side effects of therapy. Having a child on home PN may also present financial challenges by incurring additional costs on families and by impacting parental career choices due to demanding daily care required for their child, unpredictable hospitalizations and multiple appointments. Often, one parent is unable to work and cares for the child full-time. Although home PN can be very challenging for parents and children, the benefits of going home on PN include avoiding the adverse effects of prolonged
hospitalization on intellectual and social development of the child, decreased risk of nosocomial infection and cost savings for health care.\(^4\)

### 2.2.2 COMPLICATIONS OF LONG-TERM PARENTERAL NUTRITION

Parenteral nutrition is a lifesaving therapy however it is also associated with significant medical complications including intestinal failure associated liver disease (IFALD), catheter-related bloodstream infections, catheter occlusions, venous thrombosis and metabolic bone disease. \(^5\) In a review of 302 children over 20 years at a single institution in France, survival probabilities were quoted as 97\%, 89\%, 81\% and 72\% at 2, 5, 10 and 15 years, respectively. A review paper in 2006 looked at studies from Europe and the United States and quoted a similar 1 year survival rate between 91-97\% in children on home PN. \(^13\) Adult survival on home PN is thought to be 86\% and 75\% at 2 and 5 years. \(^5\) In both adults and children, it appeared that the risk of mortality is influenced by the primary disease \(^14\) as well as the potential PN-related complications experienced by the patient. In addition to medical complications, there is potential for implications on the quality of life (QOL) of both the child and their family.

### 2.3 Small Bowel Transplantation

#### 2.3.1 SMALL BOWEL TRANSPLANTATION OVERVIEW

The decision to list a patient for SBTx is complex and occurs only after a thorough evaluation of the patient's intestinal function, comorbid conditions, PN-related complications, growth and development, social circumstances and appropriateness of the surgical intervention. \(^15\) It is typically offered to patients who experience life-threatening morbidities such as IFALD, loss of venous access, recurrent catheter-related bloodstream infections and/or metabolic complications. \(^17, 18\) Following SBTx, there is significant monitoring and care required for the patient. The surgery is extensive and involves removing the diseased small bowel and replacing it with the transplanted bowel. An ileostomy is created to allow the medical team to biopsy the transplanted organ to monitor for rejection. Immediately following the procedure, the patient is intubated and monitored for hemodynamic stability in the intensive care unit. Following stabilization and extubation, the patient begins the lifelong process of rejection monitoring, immunosuppressive therapy, rehabilitation and preparation for management of their newly transplanted organ. The median hospital stay for adult and pediatric patients post-SBTx
according to the international transplant registry is between 42 and 51 days depending on the type of SBTx received. The underlying disease and range of PN-related complications determines the type of transplant that the patient receives. Patients would receive an isolated small bowel, a combined small bowel-liver transplant or a multivisceral transplant which may include small bowel, liver, pancreas, and stomach. At present, according to a survival analysis done by Pironi et al., home PN continues to be the primary choice of therapy for intestinal failure as it has a higher survival rate than SBTx and because adaptation may occur over a long period of time.

2.3.2 CARING FOR THE PEDIATRIC SMALL BOWEL TRANSPLANT PATIENT AT HOME

Kosmach et al. have examined care routines and parental perceptions of care of children post-SBTx at the Children’s Hospital in Pittsburgh. In 1997, an abstract was presented at the 5th International Symposium on Intestinal Transplant in Cambridge, UK. In this study, investigators reviewed the burden of care following small bowel transplantation in 30 pediatric SBTx recipients, although the methodology was unclear. Different time periods post SBTx were assessed including less than 1 year, 1-3 years, 3-5 years, and 5-7 years post transplant. It was noted that over time parents reported a reduction in the time required for administration of medications and feeding their child. The first year post transplant it was very common to care for a gastrostomy tube, central line and ileostomy. However, they found that tube feeding was required in only 20-25% of patients after 1-5 years post operatively and only 2 of 30 patients at 5-7 years post SBTx. The Intestine Transplant Registry 2003 report confirmed that most SBTx recipients who were alive greater than 6 months post-transplant were able to stop PN (>80%), resume oral feeding and return to normal activities, although the amount of time over which this occurred was not stated.

2.3.3 COMPLICATIONS OF SMALL BOWEL TRANSPLANTATION

Small bowel transplantation carries the potential risk of numerous complications including postoperative hemorrhage, biliary complications, vascular complications, gastrointestinal complications, acute and chronic rejection, graft versus host disease, infections, renal dysfunction, recurrent disease and graft loss requiring retransplantation. Repeated admissions post-transplant, even after the first year, are more common in SBTx than in other organ groups.
most often related to infection, dehydration, gastrointestinal complications and rejection with sepsis\textsuperscript{22}. Rejection with sepsis accounts for approximately 56\% of deaths occurring after the first year\textsuperscript{23}. Additionally, there are issues with organ availability, especially in pediatrics, and some patients die while on the waiting list. Currently, survival outcomes for SBTx range from 78-85\% at 1 year and 56-61\% at 5 years however, outcomes are improving\textsuperscript{24}. It has been shown that patients who were transplanted from home had a better survival outcome than those who were transplanted while in hospital, 67-78\% survival from home versus 51-72\% from hospital\textsuperscript{19}.

### 2.3.4 ECONOMICS OF SMALL BOWEL TRANSPLANT AND HOME PARENTERAL NUTRITION

The financial analysis of both states of health, SBTx and home PN, would be valuable however it has proven difficult to estimate the associated costs due to the individual and variable clinical courses that these patients have experienced. In 2003 Schalamon et al. investigated the mortality and economics related to short bowel syndrome and estimated that home PN typically ranges from $100,000 to $150,000 US annually\textsuperscript{25}. Although, the cost of SBTx was very high initially (estimated at $100,000 to $300,000 US)\textsuperscript{26} it was postulated that a successful SBTx would become cost-effective within 2-3 years if the patient had a functioning graft\textsuperscript{26, 27}. A more recent review by Gupte et al. in 2009 adapted information from a previously published study\textsuperscript{28} and estimated, for 3 years of home PN, a total cost $675,000 to $870,000 US compared to $360,000 for a patient 3 years post SBTx\textsuperscript{29}.

### 2.4 Quality of Life

#### 2.4.1 CONCEPTUALIZING QUALITY OF LIFE

A universal definition of QOL has not yet been agreed upon and thus many studies involving QOL are actually measuring different concepts. The World Health Organization (WHO) encompassed multi-dimensional aspects in their definition of health, which has not been amended since 1948: Health is a state of complete physical, mental and social well-being and not merely the absence of disease or infirmity. This has often been used as a basis for conceptualizing QOL with its two clear concepts: that health is multidimensional and that health is not just the absence of disease\textsuperscript{30}. In 1990, M Oleson, a registered nurse defined QOL as "the subjective perception of happiness or satisfaction with life in domains of importance to the
Anderson and Burckhardt both felt that Oleson’s multidimensional definition incorporated two important concepts that were lacking in the WHO definition: both subjectivity and perception. Quality of life was conceptualized in this study as a complex multi-dimensional concept that encompasses physical, social and emotional aspects of a person’s well-being that are important and relevant to the individual.

It is essential to clearly define the concept of QOL in order to avoid confounding with other constructs such as functional status, health status, symptoms, disease processes or treatment side-effects. In a review of 75 published QOL articles, Gill and Feinstein reported that only 15% of these studies defined the concept that they were measuring. Often functional status and health status were used interchangeably with QOL although they are not the same constructs. This is not to say that these concepts do not influence QOL, however in isolation they likely do not define an individual’s entire QOL. Also, the impact that these elements have on an individual’s QOL will be highly variable. Health status measures have traditionally focused on specific aspects of the patient’s physical illness and the assessment of their health functioning (biological or physiological dysfunction, symptoms, and functional impairment). A meta-analysis by Smith et al. determined that adults consider health status and QOL two separate concepts and that they place more value on mental functioning in relation to QOL and to physical functioning in regards to health status. Functional status assessments involve a set of questions that attempt to capture the degree of patient functioning or physical performance in order to meet basic needs, fulfill roles and maintain health and well-being. Leidy described this as functional performance and specified that this was not the same as functional capacity which is related to the patient’s maximum potential of performance. These assessments typically do not capture the subjective experience of the patient or whether these impairments impact the child in a negative manner.

2.4.2 HEALTH-RELATED QUALITY OF LIFE

Debates have ensued concerning the validity of using the term health-related quality of life (HRQOL). Health-related quality of life often refers to the inclusion of QOL dimensions relevant to health and affected by ill health; in other words, the impact of the disease and treatment on the lives of patients. Guyatt et al. defined HRQOL as those measurements of life quality that are directly related to health rather than finances or quality of environment.
Eiser and Morse extended this concept in children and acknowledged the social and emotional needs of the child’s family. However, some argue that after reviewing the adult QOL literature, the term HRQOL is simply used as a proxy for health and illness related variables. It assumes that the presence of these health and disease related variables impacts QOL for everyone, however many other dimensions of life will impact the patient’s perception of their QOL. Peter Rosenbaum provided commentary on a European study investigating QOL in children with Cerebral Palsy and he noted that it is important to separate the disorder from the person, and not make assumptions about what life must be like for that child or their family. The term QOL, rather than HRQOL, will be utilized in this study to encompass the multidimensional construct including health and illness related variables.

2.5 Measurement of Quality of Life

Quality of life is assessed in the literature using various methodologies including quantitative, qualitative and mixed methods. Ideally, QOL assessment should include both an objective and subjective evaluation. The objective component of a QOL assessment describes what the patient is able to do and feel and the subjective component assists with the translation of what this information means to the patient.

2.5.1 Quantitative Assessment of Quality of Life

Quantitative assessment of QOL includes the use of questionnaires composed of questions or statements that are evaluated by the respondent. Most scales will convert the answers into a numerical score and these scores may be further grouped into scale scores or summary scores. This allows the researcher to evaluate a numeric figure that attempts to quantify the measured concept of QOL. Kirshner and Guyatt recommend that investigators research what the original intended design of their tool was, as QOL measures typically have three main purposes: to discriminate, evaluate or predict. A discriminative QOL tool is designed to differentiate between groups of patients and typically involves items that are stable over time however, if the study requires an evaluation over time, then the tool will need to be sensitive to these changes.

Another challenge in choosing the proper QOL tool is to determine if the tool is measuring the concepts of QOL that you have chosen for your conceptual definition. It is important to be aware that many QOL tools have very different content and therefore may not actually be measuring the same concept. Results from different quantitative QOL studies may provide very
different conclusions due to different tool selection\textsuperscript{49}. Once you have selected your tools, it is essential to understand the background of your tool development as well as the testing that has been done in regards to its psychometric properties.

In a review of adult and child QOL assessments post-organ transplantation, Burra found that tools are typically assessed by item-measurement theory which examines psychometric properties of different domains \textsuperscript{30}. Psychometric properties include:

1. **Reliability** is defined by Guyatt et al. as the ratio of variability between patients to the total variability\textsuperscript{42}. In other words, it is the extent to which a measurement yields the same answer each time it is used\textsuperscript{50,51}. There are various types of reliability including intraobserver reliability (internal consistency), test-retest reliability and inter-rater reliability.

2. **Validity** is whether the instrument is measuring what it is intending to measure\textsuperscript{42}. Types of validity include face validity, content validity, and construct validity.

3. **Responsiveness** is the assessment of a tool’s ability to detect both clinically important and real changes in a specified concept over time\textsuperscript{42}.

4. **Sensibility** includes information such as the original purpose of the tool, its target population and setting, examination of the actual items, review of response options, and feasibility\textsuperscript{52}. Some aspects of sensibility overlap with face and content validity. Sensibility is the property most often missed in papers related to the psychometric properties of a tool.

### 2.5.2 QUALITATIVE ASSESSMENT OF QUALITY OF LIFE

The measurement of QOL is highly subjective and thus limiting its assessment to a quantitative tool can prevent true determination of a patient’s QOL and the meanings they associate with the items on the surveys\textsuperscript{53}. Qualitative investigation enriches data that is received from the quantitative surveys and gives a narrative description to the life experience and views of the participants\textsuperscript{47,49}. Often, health professionals make assumptions of issues they believe are important to children and families with chronic illnesses. However, the literature suggests that there is little concordance between health professionals’ perceptions and actual issues expressed by children and their families\textsuperscript{54}. When possible, attainment of the subjective impression of both the proxy and the children themselves will complement the data acquired from the quantitative
assessments. Qualitative assessment of QOL can be achieved by in depth interviews, observation, focus groups, consensus methods, case studies or with written open-ended questions.  

2.6 Questionnaires for Quality of Life Assessment

2.6.1 Disease-Specific Versus Generic Quality of Life Tools

There is significant debate surrounding the value of disease-specific versus generic questionnaires in QOL assessment. Generic measures attempt to capture overall QOL by assessing all dimensions. These tools are used for comparison across different chronic diseases and with healthy children. Generic measures however, do not address issues that may be inherent in certain chronic diseases and may not be sensitive to changes in clinical status. Disease-specific tools focus on symptoms and side-effects of a disease and its treatment and attempt to determine their impact on the individual and their QOL. These tools concentrate on issues specific to certain diseases that are not addressed in generic questionnaires. If the tool demonstrates adequate responsiveness, it can be also be used to assess changes in disease-specific QOL relating to changes in therapy, treatment, symptoms, side effects and/or disease status. The drawbacks of these tools are that they are only able to compare between individuals with the same disease and thus it is not possible to compare to different chronic diseases or to a healthy reference population. Also, disease-specific tools may not be appropriate for individuals who have more than one chronic condition. Where available, a combination of a disease-specific tool and a generic tool may enable researchers to ascertain both aspects of QOL. Currently there are no disease-specific measures for the intestinal failure, home PN or SBTx populations. A battery assessment involves the use of multiple tools in order to address the diverse characteristics of a population and may consist of both generic and disease-specific tools. Battery assessments also assist in capturing many different dimensions in multidimensional constructs, such as QOL.

2.6.2 Pediatric Questionnaires and Parent Proxy Assessment

Measurement of QOL in pediatrics presents unique challenges related to the various developmental stages encountered and the need to utilize proxy assessments. Scales used to
measure QOL in children need to consider developmental stages of children relative to cognition, emotions and ability to foresee the future\textsuperscript{60}. Questions that are developed for children must be asked in a manner that uses appropriate language and level of understanding for the child\textsuperscript{35}. As a result, QOL assessment tools require many versions for different age ranges to properly address concerns and issues in an age-appropriate manner\textsuperscript{35}. When children are too young, too ill or cognitively impaired, it is necessary to use a proxy for a child’s QOL measurement; preferably the primary caregiver who has the most intimate knowledge of the child’s well-being\textsuperscript{35}. Unfortunately this prevents the researcher from truly acquiring the child’s perception of their own QOL\textsuperscript{35}. Parents’ views and proxy assessments are impacted by a number of factors that could potentially interfere with a true assessment of what the child is experiencing. This includes knowledge of different information than the child, their own past experiences, expectations, mental and physical health, cultural expectations, educational and social backgrounds, personal knowledge of normal development and whether they have already had their child developmentally assessed\textsuperscript{61}. Parents have been found to find illness more significant than kids do\textsuperscript{35} and thus the proxy assessment may represent the fears and concerns of the proxy rather than those of the child\textsuperscript{62}. Children who are born with chronic disease may demonstrate adaptation and be unaware of what life would be like without their medical condition\textsuperscript{63}. It is common in both transplant and oncology literature, that the pediatric proxy assessments are lower\textsuperscript{36}. However, this may be related to the timing of the assessment. Parsons et al. showed in a Bone Marrow Transplant population, the children had higher QOL assessments than their parents at 6 months post transplant but this reversed at 12 months post transplant\textsuperscript{64}. When researchers compare child versus proxy mean scores on QOL assessments, a general pattern seems to arise. The proxy assessments tend to be more accurate in terms of observable behaviour e.g. physical assessment as opposed to the subjective assessment\textsuperscript{35} however in a primarily healthy population of 1105 Dutch children, the opposite result was found\textsuperscript{36}. Eiser and Morse have suggested, however, that parents have more agreement with their children when they are chronically ill rather than when they are healthy\textsuperscript{43}.

Varni acknowledges that although information provided by the proxy is often inconsistent with the patient’s own assessment, there is still crucial information retrieved from this assessment\textsuperscript{56}. Parent’s perceptions may impact the adaptation of the child, healthcare utilization, quality of
care, role in clinical decision-making, parenting practices and risk factors for complications\textsuperscript{43, 56}. The reality is that young children are completely dependent on their parents and/or primary caregivers and due to the reciprocal nature of these relationships, it remains important to measure the impact of the child’s health on the family life and to consider the view of the parent proxy\textsuperscript{61}. The parent and child views combined facilitate the development of a complete picture of the impact of disease on the child’s QOL\textsuperscript{40, 41}.

### 2.6.3 CONSIDERATIONS IN QUALITY OF LIFE INTERPRETATION

Quality of life assessment is important in scenarios where there are medical advances and when mortality is not the only primary endpoint. It can be used to assess treatment effects, treatment options, and coordination of patient support\textsuperscript{65}. Published QOL data, with its issues in conceptualization and variable methodology has resulted in a complex body of evidence. These complexities must be considered in the interpretation of QOL assessment and when interpreting current research. It has been demonstrated that QOL measurements are impacted by the timing of assessment in relation to the disease and treatment, the mode of administration, and the respondent’s mental health status\textsuperscript{35}. In addition, when respondents assess their current QOL they may be using different states for comparison e.g. pre-illness versus a prior stage of illness versus total health versus the health of friends and family\textsuperscript{35}. Comparison between adult and pediatric studies of QOL must be done with great caution. The expressed concerns of adults differ significantly from the pediatric population, except perhaps in certain adolescents. Adult QOL questionnaires often address issues such as sexual function, reproductive capabilities, financial impact and getting back to work. In 2007 Burra noted in a transplant population, that pediatric and adult measurement differed in many ways including a lack of proxy in adults, the impact of end stage disease on growth and development for children, and the requirement of immunosuppressants for a longer period of time simply due to age at transplant\textsuperscript{30}.

### 2.7 Quality Of Life in Intestinal Failure: Home Parenteral Nutrition and Small Bowel Transplant

Quality of life in home PN and SBTx populations can be difficult to assess due to the heterogeneity of the populations, the wide variation in complications, different underlying diseases, and variable post operative courses\textsuperscript{5}. To our knowledge, a direct comparison of QOL
in home PN patients to SBTx patients has been done only in the adult literature using variable methodologies and questionnaires, but not in the pediatric literature.

2.7.1 QUALITY OF LIFE IN HOME PARENTERAL NUTRITION

A 2006 study by Wengler et al. reviewed home PN monitoring practices in adults in Europe\textsuperscript{66}. They were asked whether QOL assessment was a routine procedure and if they had used a validated questionnaire. Of the 10 centres that responded, 6 reported using the SF-36 and 4 had locally developed questionnaires, however it was uncertain as to the frequency and the use of these results in the clinical setting. At the time of this study, formal QOL assessment was not routine practice in the pediatric home PN population at our institution. See Appendix A for a summary of literature examining QOL in children and adults on home PN.

2.7.1.1 The Children on Home Parenteral Nutrition

The largest study examining the QOL of children on home PN was published in the Journal of Pediatrics in 2005. Gottrand et al. performed a national, multi-centre trial assessing the QOL in children with digestive disease on home PN\textsuperscript{63}. Questionnaires were administered to patients in 5 different home PN centres and the researchers successfully received responses from 72 children, 78 siblings, 62 fathers and 68 mothers of the children. There was no description of the non-responders. The questionnaires utilized were validated, generic tools in the French language that were administered by the mail-out/mail-back method and thus were completed in the family homes. The patient and sibling questionnaires used were the Qualin questionnaire for <3 years of age, Auquei questionnaire for 3-5 years and 6-11 years of age, each completed via parent proxy, and the OK.ado questionnaire completed by children ≥12 years of age. The parents completed the Subjective Quality of Life Profile which is a validated tool in adults. The age range of the children was 1 to 19 years of age however 50% of the respondents were less than 2 years old. The data was compared to a published healthy reference group of 491 children, 268 adolescents and 90 mothers of healthy children. Overall, it was noted that the mean QOL scores were high and did not differ significantly from that of the normal population however there were some differences across specific age ranges. The parents ranked infants lower for health, eating and speaking, although the statistical significance was not stated. Children from ages 3 to 11 years had significantly lower ratings on topics including hospital, health, doctors,
medication, obligations and thinking about becoming a grown-up. The adolescents rated themselves significantly higher overall compared to the healthy cohort in QOL (mean QOL score 0.75 versus 0.54, respectively; p<0.025). They also scored themselves significantly higher in school and relations with adults (p<0.01). They scored themselves significantly lower on health (p=0.002) and sports (p=0.02). The 68 mothers ranked their QOL significantly lower than the group of 91 mothers of healthy children (p<0.001) and there was no correlation between the mother and the child’s QOL. The fathers scored themselves significantly higher than the moms relative to work, inner life and freedom although there was no healthy reference group for comparison. Additional characteristics of home PN were investigated for correlation with QOL (e.g. duration of home PN, start time of home PN, number of infusions etc.) and the only significant characteristic was related to the presence of a stoma. Interestingly, there was a negative correlation with QOL in adolescents with a stoma and a positive correlation for infants with a stoma as measured by parent proxy and no correlation in the 3 to 11 age group. The overall conclusion of this study by Gottrand et al. was that the QOL of children on home PN did not differ from healthy children. Gottrand rationalized that children and teens have effective coping mechanisms that have allowed them to become accustomed to their lives and unlike adults, many of these children had never lived a healthy life for comparison.

In Sweden in 2003, parents were asked to measure the psychological distress in their children on home PN as well as the impact of having a child on home PN on themselves. Twenty one of 25 home PN families participated by answering a non-validated questionnaire about HPN, the Child Behavior Checklist (CBCL - 118 behaviour problem items on a three point scale), as well as the Interview Schedule for Social Interaction (30 questions about social network and social support of the parents). Engstrom et al. found that the parents ranked their children as having much lower social activities, less friends and worsened school performance. In addition, they felt their children demanded a high level of attention, displayed rapidly changing emotions and complained of non-specific bodily pain. These scores were compared to published data and found to be similar to inflammatory bowel disease children but worse than children with diabetes and chronic tension headache. The authors concluded that these children and adolescents on home PN were psychologically distressed however were uncertain of the reason.
An online qualitative study published in June 2004 by Heidi Silver looked at adult patients from the United States on home PN across many age groups as well as mothers and their three children (aged 8, 8, and 10 years). They participated in a 2-3 hour interview via online instant messaging. The children and their mothers expressed fears regarding the complications of being on home PN, most specifically related to the development of IFALD. Other issues included the development of central line infections and loss of venous access. In this study, the three children reported that they participated in similar activities as their peers and reported that the main way that they felt different from their peers was due to the difference in their growth. Each of these 3 children was the shortest in their class. In this study, hunger was expressed as a critical issue in adults but not in children. However, the children reported that social situations where eating was a central theme as highly stressful e.g. holidays due to the pressure to eat from parents and healthcare providers rather than an unfulfilled desire to eat. This was a small study that acknowledged the potential bias that the methodology mainly accessed patients who were within a higher income and education level as demonstrated by access to internet.

2.7.1.2 The Parents and Families of Children on Home Parenteral Nutrition

The British Artificial Nutrition Survey (BANS) requested questionnaire data from 81 families from 1996 to 1999 and had a 38% response rate. Common complaints included sleep disturbance, deterioration in family life, poor social life activities and poor overall QOL in families of children on home PN. In an unpublished study, Wong et al. determined that having a child on home PN had a significant impact on the QOL of the parents. They used a mixed methods design of focus groups, semi-structured interviews, and 2 questionnaires (General Health Questionnaire (GHQ-28) and the British Artificial Nutrition Survey (BANS)). They determined that 7 of 11 parents exceeded a threshold for psychiatric morbidity on the GHQ-28. More specifically from the BANS, the parents experienced deterioration in family, sex, social life and work life when compared to controls. Due to the intense management of home PN required, these parents were often unable to take holidays and spend time with their partners. They admitted to feeling frustrated, annoyed, stressed and had difficulties sleeping. Parents in the Swedish study by Engstrom et al. also reported issues with their own attachment, relationships with their other children, and spouse, family and social integration. Vanneste et
al. conducted a pilot study of 10 mothers who had children on home PN and assessed anxiety levels using a validated tool, the Max Hamilton Anxiety Scale. They determined that anxiety was the highest at diagnosis, was reduced once they were trained in delivery and minimal at follow-up visits when they demonstrated more confidence with PN technique. However, there was an increase in anxiety associated with the child having a poor prognosis. Other factors that had a negative impact on anxiety included job renunciation and an unsettled future, whereas continued care and close follow-up had a positive influence on confidence and anxiety.

Margaret Rhone et al. completed a study in 1986 at the Hospital for Sick Children and surveyed parents of children on home PN and home enteral nutrition to determine coping strategies. The mothers, whom were the primary caregivers, reported feeling run down, isolated and time-pressed. They expressed concern surrounding their child’s future and worried about the effect of the illness on the other children in the family. Similar themes were found in a British nursing study published in 2005 by Sexton et al. which reviewed the current state of homecare for pediatric patients on home PN using one-on-one interviews in 20 homes. The following themes were revealed upon content analysis: physical exhaustion, psychological issues related to having home care and the burden of care of having a child on home PN. In addition, the parents confirmed their fear for the future of their children. In the Engstrom study from Sweden, parents were assessed for perceived pressure using a non-validated method. A single question was asked of 21 parents: Do you abstain from wine or similar alcohol beverage because of your responsibility for connecting/disconnecting your child to PN? Ten of 21 parents responded yes, 11 responded no and consequently the team concluded that there was evidence of pressure on the families. In the online instant messaging study by Silver in 2004, the mothers of the three children involved in the study expressed having an initial terrifying fear of having a child on home PN, feared the potential complications like their children but also felt that this was what gave their child a chance at life. Consistent with adults on home PN that were also interviewed, the mothers expressed that it was very stressful to find their own source of support.
2.7.1.3 Adults on Home Parenteral Nutrition

A systematic review published by the Huisman-de Waal in 2007 reviewed the impact of home PN in daily life and found 22 studies that investigated QOL in adults. Ten of these studies utilized generic QOL questionnaires and 12 studies used other questionnaires and interviews. The results were inconsistent however in summary, QOL was lower than that of the healthy population but was similar to others who were on nutrition support and had end-stage renal disease (ESRD). The same author performed an open interview study the year prior in 48 adults to answer one specific research question: “What is the nature and context of the problems experienced by patients as a result of receiving home PN in the home situation?” Qualitative analysis revealed 7 themes including negative emotions, physical problems, social limitations, dependence on others, incapability, complications, and patient provider problems. In contrast, Winkler completed 3 open narrative interviews in adults and determined less negative responses compared to previous literature. The themes identified in this much smaller population included the view of PN as lifesaving therapy, adaptation, quality of life, sustenance and security and some negative issues related to food and eating. This was attributed to the fact that all 3 of the patients received PN as life-saving therapy and were very appreciative of its effectiveness and their chance for life.

Richards et al. used the Short Form (36) Health Survey (SF-36) to compare adult home PN patients with the standard population and showed that 6 of 8 domains were lower than the standard norms. However, when these respondents were separated into younger (< 45 years) and older (> 55 years) age groups, the younger group’s assessment of their QOL was significantly better than the older group and approached the levels of the norms. In 1999, Jeppesen published a study in Gut looking at 49 adults on home PN and compared these to 36 non-home PN patients who suffered from short bowel syndrome. He utilized a generic tool which was a measure of adult functional status (the SIP - Sickness Impact Profile) and a disease-specific tool validated for the inflammatory bowel disease population (the IBDQ). The home PN patients scored worse on both questionnaires in comparison to the non-home PN patients. In addition, they found that females scored worse than males and that those individuals who were older (> 45 years) had poorer scores which confirmed the results of the earlier study by Richards in terms of the effect of age. Jeppesen’s final conclusion was that adults on home PN
experience a QOL that is comparable to pre-published SIP data of adults with ESRD on dialysis. Margaret Malone studied QOL at 2 time points, 3 years apart, in seventeen patients (13 were on home PN and 4 were on home enteral nutrition)\textsuperscript{77}. Scores on the SF-36 were lower than population norms at both time points and did not change significantly over the 3 year time period. A non-validated lifestyle and activity questionnaire for patients on home PN that had been previously used as an interviewing tool\textsuperscript{78}, identified the most prevalent issues as sleep disruption, difficulties with travel, and limitations in social life and choices of entertainment\textsuperscript{77}. Chambers examined QOL in adults over time using the SF-36\textsuperscript{79} and they noted that QOL appeared to improve from the immediate post-discharge period on home PN to 6 months post-discharge but then the effect seemed to taper off.

2.7.2 QUALITY OF LIFE IN SMALL BOWEL TRANSPLANTATION

Quality of life has been investigated in the SBTx population in both adults and children using multiple comparison groups including healthy norms, ESRD, diabetes and liver transplant. In the adult literature, home PN has been directly compared to SBTx, however there was no published data in children. Quality of life is not formally considered as listing criterion for SBTx. Consequently, children who do not develop life-threatening medical complications despite having significant PN-associated morbidities may be maintained on home PN indefinitely\textsuperscript{11}. Beath et al. proposed a definition of “Impaired Quality of Life” for the home PN population in the May 2008 edition of Transplantation to assist with its inclusion in listing criteria\textsuperscript{17}. The definition of poor QOL consisted of 3 criteria: 1. by self-assessment in a validated semi-quantitative questionnaire; 2. prolonged hospitalization (more than 4 weeks in any 1 year) or recurrent hospitalizations (more than 4 admissions per year or 12 total admissions involving at least one night in hospital) and 3. pain intense enough to require regular narcotic analgesics. This has not been formally included in listing criteria for many institutions, including the Hospital for Sick Children and should be further validated before implemented in practice. See Appendix B for a summary of literature examining QOL in children and adults post SBTx.

2.7.2.1 The Children Post Small Bowel Transplant

In 2000, Sudan and Iverson assessed function, growth and development in 31 patients post SBTx, with an equal distribution of males and females\textsuperscript{80}. Twenty-seven of these patients were
children and 4 patients were adults with a mean length of follow-up of 39 ± 20 months. The study was descriptive and used a non-validated questionnaire that included a QOL assessment. Quality of life was defined using the number of hospitalizations or illnesses, presence of an ostomy and number of bowel movements per day. These are markers that may impact QOL but did not represent the multidimensional concept of QOL. They concluded that almost all of the patients were off PN, demonstrated good growth velocity, experienced few hospitalizations (2.3 ± 1.1 during the follow-up period) and returned to work or school. There is little known about school performance after transplantation and data is mostly extrapolated from assessments of overall QOL. Thevenin et al. performed numerous neurodevelopmental tests to assess presence of developmental delay in children and infants post intestinal and multivisceral transplant. He determined that the children post SBTx had more cognitive delays than children who had received an isolated liver transplant and that infants post SBTx continued to suffer significant cognitive delays several years out from transplant. Children with a multivisceral transplant may have also had a higher risk of developing or continued developmental delay compared to those who had an isolated bowel transplant.

In 2004, Sudan et al. assessed QOL in pediatric patients post SBTx using a generic questionnaire and compared them to published norms and published data of children with ESRD on dialysis, diabetes and post liver transplant. They did not compare these patients to children on home PN. Their primary outcome was defined as the comparison to published norms because restoration of complete health was the goal of SBTx. Twenty-one children and 22 parents were surveyed with the Child Health Questionnaire (CHQ) via mail-out/mail-back process, resulting in a 76% response rate. The mean age was 11 years, 9 were male and 13 were female. Recipients between ages 5-10 were scored significantly lower via parent proxy than the older children (>10 years) in regards to global health, general health perception and family activities. This study did not find significant differences in the children’s self-assessment post SBTx when compared to published norms. In some cases the SBTx recipients ranked themselves higher than the published norms, although only mental health reached the level of statistical significance. Mental health was also ranked significantly higher in comparison to children with ESRD on dialysis. The patients with SBTx ranked themselves a minimum of 10 points higher (out of a possible 100) than the ESRD children in most domains however; these did not reach statistical significance. Some individuals have suggested that children are able to
adapt well and likely have less recollection of pre-transplant complications or experienced post-operative pain\textsuperscript{26}. This was potentially linked to the high rate of non-compliance for medication that has been documented in teenagers who were post kidney and/or liver transplantation\textsuperscript{26}. This occurrence was not known for patients who were post SBTx. The Sudan study was composed of a small sample size and was powered to detect a 20 point difference\textsuperscript{18} which may have precluded detection of further differences. An abstract presented by a group from UCLA in September 2009 in Italy at the Small Bowel Transplantation Symposium compared QOL in pediatric SBTx patients using 2 validated proxy instruments \textsuperscript{1} the CHQ-PF50 and Varni\textsuperscript{\textregistered}'s PedsQL 4.0\textsuperscript{TM}\textsuperscript{83}. The data on the 33 SBTx patients was compared to a healthy population and to published pediatric liver transplant scores (35 post liver transplant proxies completed the CHQ PF-50 and 77 completed the PedsQL4.0\textsuperscript{TM}). The CHQ-PF50 proxy assessment demonstrated that patients with SBTx scored lower than the liver transplant population in parental impact on time, activities, emotional role and behaviour. The PedsQL4.0\textsuperscript{TM} also showed a lower score on physical health, social and school function. The SBTx patients scored lower than healthy children on all previously mentioned domains and additionally physical function, physical role, general health, emotional impact on parents, emotional function and psychosocial health. This study also attempted to delineate predictors of a lower QOL score in SBTx patients and found that, using the CHQ scores as the primary outcome, the predictors included: single care provider, worse nutrition and renal status, the presence of devices (e.g. feeding tube, ostomy, or central line) and an intravenous fluid or PN requirement. The latter predictor was also implicated in a lower QOL score on the PedsQL\textsuperscript{TM}\textsuperscript{83}.

The parent proxy assessment in the Sudan study was completed primarily by moms (21 female versus 3 male respondents) and their assessments trended towards a lower ranking of these children compared to how the children ranked themselves\textsuperscript{18}. However, the differences in assessment of general health perception and physical role limitations were the only two that were statistically significant. In contrast to the parents of healthy children, the parents of children post-SBTx rated their children significantly lower in 3 domains \textsuperscript{1} physical functioning, role/social limitations owing to physical function, and general health. Sudan et al. suggested that parents may be biased due to their own emotional stress related to their child\textsuperscript{\textregistered}'s illness\textsuperscript{18}. The oncology literature explains this phenomenon in relation to parental anxiety\textsuperscript{36}. 

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The Parents and Family of Children Post Small Bowel Transplant

Sudan et al. found that the parents of children post-SBTx perceived a **negative impact on emotion, time and on family activities**. Kosmach et al. have examined care routines, parental perceptions of care as well as parental psychosocial outcomes at the Children’s Hospital Pittsburgh. In 1997, two abstracts were presented in Cambridge UK at the 5th International Symposium on Intestinal Transplant. The first study resulted in 66% of the parents stating that **caring for their children was easier post-SBTx** than pre-transplant. The second abstract attempted to measure the mental health and stress of the parents of 10 children post-SBTx, although the methodology was unclear. The results demonstrated **normal mental health scores but high scores in depression, hostility, and obsessive and compulsive elements**. It was noted that the majority of the stress was observed in the acceptability of and demands of the child. This same group from Pittsburgh expanded this data and performed a cross-sectional study of parental psychosocial outcomes in both pediatric liver and/or small bowel transplantation. They compared 30 parents (20 mothers, 10 fathers) of children at referral for transplantation to 31 parents of children who were 2 months post-SBTx (21 mothers, 10 fathers). They measured mental health using the BSI Brief Symptom Inventory giving a global index of distress, parenting stress by the PSI Parenting Stress Index which assesses stress in the parent-child relationship for families of kids 12 years of age and younger and parent’s QOL using the SF-36. In addition they examined potential predictors of parental psychosocial outcomes using demographic factors, a functional status assessment of the child’s adaptive behaviours (using Vineland Adaptive Behaviour scales), the family environment scale and parental coping assessed by the Child Health Inventory for Parents (CHIP). Forty-two of 102 possible families participated, 24% were eliminated due to language barriers. There was a suspected survival bias because 14% of patients had died. Parents reported **increased psychological symptoms** with **fathers rating higher symptoms than moms**. Parenting stress was not statistically higher than the published norms but did show an increased trend which was consistent with other parenting stress literature related to chronic illnesses. Parents experienced higher stress in the presence of an elevated family conflict score and with a younger child undergoing transplantation. Parents scored lower on vitality however did report good physical health. This pilot study was stated to have demonstrated that **psychosocial adjustment did not differ with point of transplantation process** however they only examined the early SBTx postoperative period. Longitudinal
studies are required to better determine the impact of the time of SBTx on psychosocial adjustment.

A recent abstract published at the 2009 International Small Bowel Transplant Symposium from the Birmingham Children’s Hospital in the United Kingdom described QOL in parents of home PN patients (n=4) and compared them to parents of pediatric patients who were post-SBTx (n=12). They used the CHQ-PF50 and the General Health Questionnaire (GHQ) at one time point for the home PN parents and at 3 time points for the SBTx parents (pre SBTx, 6 months and 12 months post SBTx). They only used the parental and family impact scales on the CHQ-PF50 as there were no reported results on proxy perception of the child’s QOL. The parents whose children were awaiting a SBTx had lower GHQ scores compared to the parents of the kids on home PN which related to an increase in somatic symptoms, anxiety/insomnia, social dysfunction and depression. Post SBTx, the GHQ scores did improve, as did the CHQ-PF50 scale impact on parental emotion however there was no improvement on parental impact on time. Due to the small sample size, it was difficult to determine whether the mental health of SBTx parents was different than parents of children on home PN.

2.7.2.3 Adults Post Small Bowel Transplant

Dimartini et al. in 1998 published a cross-sectional QOL study of 9 post SBTx patients and 10 adult patients on home PN. The response rate was 75% in the SBTx group and 55% in the home PN group. Quality of life was measured using the QOLI (Quality of Life Inventory), which is a disease-specific tool designed for transplant patients and was only validated in adult liver transplant patients. All 19 subjects were asked to retrospectively rate their QOL in their pre-morbid/pre-PN state (this was when the patient was in good health or during a period of chronic illness). The 10 subjects that were currently on PN were then asked to evaluate their current morbid state on home PN. The 9 subjects who were post SBTx were asked to retrospectively evaluate their morbid state on PN and also their current state post SBTx. Both groups ranked their QOL once starting home PN significantly worse than their pre-PN state. In fact, the current home PN group felt that initiation of home PN had significantly worsened anxiety, depression, drug use, pain and discomfort, stress, digestive and urinary symptoms, loss of control, and decreased physical mobility, energy, optimism, and sleep (p<0.05). Overall the post SBTx group noted a significantly improved QOL post transplant compared
to their time on home PN in most areas without a worsening in any areas. However, when comparing their pre-morbid/pre-PN state to their post SBTx state, there was a significant worsening in the need for more meds and drugs, decreased mobility, increased pain and discomfort, difficulty parenting, poor sleep, loss of control and poor quality of social support. Rovera used similar methodology in 1998 and asked 10 SBTx patients (mean 2.7 years after SBTx and a mean period of 5.3 years of intestinal failure) to retrospectively evaluate their lives before illness, during illness (on home PN) and post SBTx using the same tool, the QOLI\textsuperscript{88}. They also asked 10 home PN patients (mean period of 5.1 years of intestinal failure) to evaluate both their previous and current QOL. A significant improvement was noted in most areas of QOL after transplantation which favourably compared to their pre-illness state. In this study, the QOLI was readministered after 2 years in 4 patients from each group (n=8) and found that QOL improved over time after SBTx and resulted in decreased anxiety over physical functioning. This study differed from many other adult studies because of the inclusion of patients with liver failure which is less reflective of the adult population of patients on home PN. These two studies by Dimartini\textsuperscript{87} and Rovera\textsuperscript{88} concluded that life post SBTx is better in many patients compared to life on home PN or at minimum, not worse than life on home PN\textsuperscript{26}. The QOLI was not validated in these populations and the reliability and validity was compromised due to the combined cross-sectional/retrospective nature of the studies. On the contrary, providing patients with a state of comparison may have been beneficial for assessing change in QOL. An improved design, as Rovera attempted\textsuperscript{88}, would have been to use a responsive QOL tool to prospectively evaluate changes in QOL over time however this would not be feasible with a small patient population.

In 2006, Pironi used the SF-36 to compare QOL in 12 post SBTx patients, a minimum of 9 months post transplant and 18 home PN patients, a minimum of 9 months on home PN\textsuperscript{89}. The home PN patients were significantly older (p=0.031) than the SBTx patients by approximately 10 years and there were more females in the home PN group. Three of the 12 SBTx patients were receiving intravenous fluids 3 to 6 days per week. The only significant difference between the two appeared to be that of the bodily pain score which was worse in the home PN patients than the SBTx patients. However there was a non-significant trend with the home PN patients scoring lower in all other measured domains. Pironi concluded at the end of the paper that perhaps in a few very select patients on home PN whose QOL was significantly impaired, a
referral could be made to a SBTx center and the patient could be assessed and allowed to make an informed choice. In 2007, ÓKeefe looked at the relationship between good allograft function and a positive view of QOL in 46 adult SBtx patients and found a positive correlation\textsuperscript{90}. The QOL tool was unspecified however the instrument contained 26 domains and 130 questions and was designed for the liver transplant population (potentially the QOLI). They administered the QOL questionnaire pre and post transplant to all surviving SBTx patients (34 of 46 patients transplanted) and also compared these results to 13 home PN patients not listed for transplant. The QOL of patients on home PN not listed for transplant was similar to patients who were pre-SBTx. However, once the patient received a SBTx there appeared to be a significant improvement in QOL, with higher QOL scores than patients who were on home PN but not listed for transplant. The results were difficult to define but it appeared that QOL improved post SBTx in patients who did pre and post QOL questionnaires.

In 2002, Cameron attempted to answer the question of whether or not QOL depended on patient acuity when on home PN. He surveyed 3 groups of adult patients: 4 patients post SBTx, 12 patients stable on home PN and 6 patients on home PN who had been assessed for SBTx but did not get listed (i.e. complicated intestinal failure)\textsuperscript{91}. Two generic questionnaires were used: the Nottingham Health Profile and the SF-36 (one SBTx patient did not complete the SF-36). There was no significant difference between overall QOL in patients who were post SBTx and those who were stable on home PN. However, QOL was significantly worse for those with complicated intestinal failure compared to those stable on home PN in terms of energy, emotional reactions, physical function, and social functioning. The author suggested that the patients who had complicated intestinal failure were a better comparison group than those patients who were stable on home PN\textsuperscript{91}. Golfieri et al. measured the psychological well-being and QOL in 27 adult SBtx patients between March 2004 and May 2009 and compared them to a control group of 27 healthy subjects \textsuperscript{92}. A battery assessment was used and the transplant patients scored lower in the psychological domain, positive relations and autonomy but scored higher in terms of personal growth. For patients with > 8 admissions, there was evidence of increased anxiety, depression and somatic symptoms. There did not appear to be significant differences related to the number of rejections or sociodemographic variables. Golfieri et al. confirmed that SBTx patients had a long period of adaptation in their role as a transplant recipient\textsuperscript{92}. 

\textsuperscript{24}
Chapter 3
Rationale, Aims, Research Question, Hypotheses and Objectives

3.1 Study Rationale

Important advances in the management of intestinal failure and the implementation of a multidisciplinary intestinal rehabilitation program at The Hospital for Sick Children have resulted in improved survival, reduced IFALD and consequently more patients on long-term home PN for extended durations. Enhanced survival is often accompanied by increased health care needs related to the patient’s chronic condition and thus QOL issues become more important. These patients suffer significant short-term and long-term morbidities such as repeat hospitalizations, recurrent sepsis, cholestasis, intestinal discomfort and pain. Caregivers of these children are required to provide complex care including the management of ostomies, high stool output, intravenous access, and feeding tubes. Feeding and care regimens are complicated and time-consuming, necessitating persistent vigilance in monitoring and response to potential life-threatening side effects of therapy. Families are forced to balance domestic, family, relationship and employment responsibilities and as a result, often experience physical and emotional exhaustion. All of these aforementioned issues have the potential to impact QOL for patients, their caregivers and families. As survival outcomes improve for SBTx, deteriorating QOL pre-transplant may become an important consideration as part of the listing criterion. In 1999 Jeppesen stated, “as the results of intestinal transplantation will probably improve in the coming years, this procedure may become an alternative to home PN on the lines of renal transplantation and dialysis, not only on vital indications, but also with the aim of improving QOL in these patients”. After completing a Markov Analysis, Lopushinsky et al. stated that QOL should be included when considering transplantation. They demonstrated that when considering quality-adjusted life years, early transplantation was considered the preferred strategy versus the standard care even while considering a broad range of clinical variables and utilities. Quality-adjusted life years (QALYs) is a value that is placed on a certain life state relative to death or perfect health to produce a preference based utility in relation to the duration of a health state. Limitations were acknowledged in their long-term predictions due to a paucity of data as the majority of improved outcomes are within a 1-2 year period post SBTx.
To our knowledge, there were no published studies using mixed methods to assess the QOL of pediatric patients with intestinal failure on home PN and their caregivers; nor were there studies using validated instruments comparing children on home PN to children post SBTx. Quality of life assessment in children can promote understanding the experience of the illness. Healthcare professionals need to be aware of these experiences and challenges so that appropriate support systems, services and interventions can be implemented for these children and their families. Long-term home PN is a tremendous challenge for both patients and their families and it is essential to have access to valuable and cost-effective supports to optimize positive outcomes of physical, emotional and social health.

3.2 Aims

Primary aim
1. To assess quality of life in pediatric patients with intestinal failure on home parenteral nutrition using a mixed methods approach.

Secondary aims
1. To compare quality of life in pediatric patients with intestinal failure on home parenteral nutrition to age-matched population norms and patients with intestinal failure who have received a small bowel transplant.
2. To describe the impact of having a child on home parenteral nutrition on the primary caregiver and their families.

3.3 Research Question

What is the quality of life in pediatric patients with intestinal failure on home parenteral nutrition compared to published population norms and patients who have received an intestinal transplant?

3.4 Hypotheses

Primary Hypotheses
1. Quality of life in pediatric patients with intestinal failure on home parenteral nutrition is lower than published age-matched healthy norms.
2. Quality of life in pediatric patients with intestinal failure on home parenteral nutrition is lower than published patients post small bowel transplant.
Secondary Hypotheses
1. Intestinal failure patients on home parenteral nutrition will demonstrate reduced functional status, lower overall quality of life, lower overall general health and increased pain versus patients in our institution who have had a small bowel transplant.
2. Quality of life, as measured by a generic tool, in pediatric patients with intestinal failure on home parenteral nutrition is lower than that of patients post small bowel transplant at our institution.
3. Family function will be lower in families of children on home parenteral nutrition compared to post small bowel transplant patients at our institution.

3.5 Objectives

Primary Objectives
1. To assess quality of life in children on home parenteral nutrition with intestinal failure using a generic quantitative measure and to compare this to published norms.
2. To compare quality of life in children on home parenteral nutrition with intestinal failure with that of published pediatric small bowel transplant patients.
3. To describe the experience of home parenteral nutrition in children with intestinal failure and their families using qualitative methodology.

Secondary Objectives
1. To compare quality of life in children with intestinal failure on home parenteral nutrition to children post small bowel transplant from our institution using a quantitative battery assessment.
2. To describe the impact of having a child on home parenteral nutrition on primary caregivers and family using the Family Impact Module.
Chapter 4
Methodology

4.1 Research Design

The study design was an observational, cross-sectional, mixed-methods analysis of QOL in pediatric patients with intestinal failure on home PN utilizing both standardized generic QOL questionnaires and qualitative focus groups. A mixed-methods design was chosen as it was felt that both quantitative and qualitative measures provide different but essential and complementary information. The results of the questionnaires were compared to published population norms, published SBTx data, and institutional SBTx data. According to the framework proposed by Kirshner and Guyatt, the purpose of our chosen instruments was to be descriptive/discriminative as these are effective in cross-sectional analyses where there is a need to distinguish the burden of morbidity between groups. The qualitative research arm included focus groups to enrich the data by exploring and clarifying the parent and child experiences in further detail, to explore information elicited by the questionnaires, and to obtain perspective of QOL on home PN that the questionnaires were unable to acquire.

4.1.1 SETTING

The study population was recruited from GIFT (Group for Improvement of Intestinal Function and Treatment) and Multi-Organ Transplant Centre outpatient clinics at the Hospital for Sick Children (SickKids) in Toronto, Ontario. Following institutional ethics approval in December 2009 (See Appendix C), questionnaires were administered in the outpatient clinic setting and the focus groups were conducted in a conference room within the same institution.

4.1.2 DESCRIPTION OF THE POPULATION

Study participants included children with intestinal failure on home PN and post SBTx, as well as their parent proxies. These children were followed by the GIFT team and/or multi-organ transplant program at the Hospital for Sick Children. GIFT has managed 165 patients over the last 7 years since the team’s creation in 2003. Currently, there are approximately 25 patients on home PN that are followed by the GIFT team and approximately 7 children post SBTx who are managed by the multi-organ transplant team.
**GIFT Usual Care:** The patients with intestinal failure at SickKids are followed by the GIFT team which is a multi-disciplinary team represented by general surgery, gastroenterology, neonatology, transplantation, clinical nutrition, nursing, social work, rehabilitation and palliative care. The goals of GIFT are to promote intestinal rehabilitation, wean children from PN, provide integrated care and improve communication between medical teams that care for these children, and to engage in research to advance care for children with intestinal failure. Follow-up frequency is variable depending on the child's clinical status, home PN duration and enteral and parenteral nutrition tolerance and progression. These children are managed in regards to fluid and electrolyte status, nutritional status and growth, social issues and development.

**SBTx Usual Care:** The SBTx team is part of the integrated Multi-Organ Transplant Centre at SickKids. The children are monitored very closely post-transplant. Small bowel biopsies are conducted via stoma two times weekly up to a minimum of every 2 months until stoma closure. Bloodwork and clinic visits vary depending on laboratory and procedural findings, changes in immunosuppression, and the child's clinical condition. They range from twice weekly immediately post discharge, every 2 weeks between 3 and 6 months post SBTx, every month from 6 months to 1 year post SBTx and finally every 3 months after 1 year post SBTx or as clinically needed. The children are managed closely in terms of immunosuppression, fluid and electrolyte status, nutritional status and growth as well as social issues and development.

### 4.1.3 ELIGIBILITY CRITERIA

**Inclusion Criteria**

- Children on home parenteral nutrition with intestinal failure secondary to underlying gastrointestinal etiology (as per GIFT guidelines, adapted from the Canadian Association of Pediatric Surgeons case definition for short bowel syndrome\(^1\) having <25% of predicted bowel length for gestational age OR remaining on parenteral nutrition for >42 days secondary to bowel injury or disease)
- Children with intestinal allografts (either isolated small bowel or multivisceral transplant which includes an intestinal allograft) a minimum of 6 months post-transplant (following the highest-risk period)
- Age of 2 months (corrected if premature) to 18 years at time of questionnaire administration
Exclusion Criteria

- Patients on home parenteral nutrition secondary to non-GI underlying disease states (e.g. malignancy, progressive genetic disorders, bone marrow transplantation, cystic fibrosis etc.)
- Inability to provide informed consent or to complete questionnaires due to language or severe developmental barriers (child excluded only).

4.1.4 POTENTIAL RESEARCH SUBJECTS

Potential research subjects were identified via patient lists and chosen according to eligibility criteria. The intestinal failure patients were generated from the patient list maintained by the GIFT team and the SBTx patients from the pre-existing electronic transplant patient database. Initial contact was made via the responsible physician in the GIFT and transplant clinics.

4.1.5 PHASE 1: QUANTITATIVE COMPONENT (SURVEY ADMINISTRATION)

Once patients were identified according to eligibility criteria, they were assigned a unique identification (ID) number which was recorded by a co-investigator and kept in a password protected document on a password protected computer in a locked office. The ID number was documented on all questionnaires and data collection forms. Basic demographic data was collected for all patients that met eligibility criteria in order to compare respondents with non-respondents (See Appendix D – page 1). The clinic physician known to the family introduced the study to the child and family. If they expressed interest, the physician arranged for a co-investigator to speak with them to describe the study in further detail and to acquire informed consent/assent. If the child/family did not agree to participate, no further contact was made by the research team.

After informed written consent was obtained (See Appendix E - Consent Forms for Quantitative Arm of Study), the children and parent proxies were given the battery assessment of questionnaires for completion during the clinic visit preferably prior to their appointment or to be taken home depending on preference and time constraints. Sudan et al. reviewed the literature and found that response rates were highest when the questionnaire was administered at the time of the clinic visit and second highest on the telephone. If families were unable to complete the questionnaires in clinic, they took the package home with a pre-paid postage return envelope and
were given contact information of the co-investigator if questions arose. These were returned to the hospital upon completion. Families that lived out of province or did not have a scheduled clinic appointment during the recruitment period were contacted by the primary physician. If the family demonstrated interest in participation, the battery assessment was mailed to their home with a pre-paid return envelope. The children and adolescents were encouraged to complete the measurement tools independently of the parents and if they required assistance, a co-investigator was available in clinic or on the telephone to answer questions. The PedsQL4.0™ Self-Report for Young Children (Ages 5-7) was administered by a co-investigator in the clinic setting. Once the questionnaires were completed and detailed medical background information was collected by a co-investigator (See Appendix D pages 2-3), data was entered into a password-protected excel spreadsheet with unique ID and maintained in a locked office.

4.1.6 PHASE 2: QUALITATIVE COMPONENT (FOCUS GROUPS)

The primary caregivers of children followed by the GIFT who had agreed to complete the questionnaires were considered for inclusion in the focus group. The ideal size of a focus group according to Kitzinger is between 4 and 8 individuals. Morgan stated that for patients or families with a higher level of emotional involvement in the subject matter, small groups were essential. We aimed to recruit 5 to 6 proxies from the home PN population to participate in a 1.5 to 2 hour semi-structured focus group. We also aimed to recruit 2 to 3 children on home PN to participate in a semi-structured focus group for approximately 1 hour duration. The children’s focus group size target was smaller because there were few children in the GIFT program old enough to participate in such a forum.

Purposeful sampling was used for the parent focus groups to achieve maximum variation of broad and diverse perspectives of various age groups and durations of home PN. We restricted focus groups to families who lived locally unless they were willing to travel longer distances to participate in the focus group.

The focus group was conducted face-to-face at the Hospital for Sick Children with beverages and snacks available. Families who came to Sick Kids to be interviewed were reimbursed for costs incurred for transit and parking to a maximum of $20 per family. The primary facilitator of the focus group was Julia Maxwell, M.S.W., an experienced Social Worker in the GIFT and SBTx.
programs at the Hospital for Sick Children. The co-investigator, Megan Carricato, assisted with audio taping of the sessions and probing questions. A trial focus group was conducted with the SBTx proxy population. Nicole deSilva, an experienced qualitative researcher and nurse practitioner in GIFT and neonatology acted as a co-facilitator during the SBTx focus group and provided feedback in preparation for the home PN focus group. A list of questions was provided to help to guide the discussion in the focus group (See Appendix F – Focus Group Preparation). Written consent for this aspect of the study was explained and obtained separately from the questionnaires at the initiation of the focus groups (See Appendix G - Consent Forms for Qualitative Arm of Study).

4.2 Main Outcome Measures

4.2.1 QUANTITATIVE OUTCOME MEASURES

The battery assessment contained standardized measures of QOL, functional status, overall health and pain, and differed according to each age group and whether it was proxy or self-administered. Quality of life is a multidimensional concept and there was no single measure that was able to capture all aspects of the construct. Therefore, a battery approach was used with a combination of validated tools to help capture relevant dimensions. The tools were reviewed according to Taylor et al. who considered five aspects in determining suitability for use: the content of the questions, the respondent, age appropriateness, validity of the translation and the psychometric properties.

The generic measures of QOL were developed in the United States by multiple authors including the Child Health Questionnaire by Jeanne Landgraf and the PedsQL4.0™ by James Varni. Both tools were developed utilizing the 1948 World Health Organization (WHO) definition of health as the underlying concept for QOL: “Health is a state of complete physical, mental and social well being and not just the absence of disease or infirmity.” This conceptual definition was utilized as a basis for the underlying construct of QOL in this research as well with the inclusion of perception and subjectivity.

Child-Completed Measurement Tools
See Appendix H for a list of the self-completed child measurement tools utilized in this battery assessment.

1. The Child Health Questionnaire Child Form (CHQ-CF87) is a self-completed, 87 item general measurement of QOL for children age 10 to 18 years\textsuperscript{97}. This survey includes a subjective assessment of the following domains: i) General Health; (ii) Physical Functioning; iii) Everyday Activities; iv) Pain; v) Getting Along/Behaviour; vi) General Well-Being; vii) Self-Esteem; viii) Your Health; and ix) You and Your Family. This survey was previously published in SBTx literature allowing for comparison with the data collected in this study.

2. Pediatric Quality of Life Inventory (PedsQL4.0\textsuperscript{TM}) is a self-completed tool with 23 items to measure the QOL for children aged 5-7, 8-12 and 13-18 years. There are 23 items and 4 domains: Physical, Emotional, Social and School Functioning.

3. Visual Analogue Scale (VAS) is a straight line with anchors on each end signifying extreme measures of a sensation, feeling or response (e.g. 0cm=worst QOL, 10cm=best QOL)\textsuperscript{99}. Children (ages 8 to 18 years of age) were asked to mark the VAS relating to overall QOL, general health and pain\textsuperscript{99}. The VAS in this study was a previously validated 10cm continuum which was used to measure each of the aforementioned concepts over the preceding 3 months\textsuperscript{100}.

**Parent Proxy Measurement Tools**

See Appendix H for a list of proxy-completed measurement tools utilized in the battery assessment.

1. One of two possible questionnaires by Jeanne Landgraf was used depending on the age of the child (CHQ-PF50 or ITQOL). The Child Health Questionnaire Parent Form (CHQ-PF50) is a parent proxy general QOL survey for children aged 5 to 18 years\textsuperscript{97}. This 50-item form included the assessment of domains including: i) Global Health; (ii) Physical Activities; iii) Child\’s Everyday Activities; iv) Pain; v) Behaviour; vi) Well-being; vii) Self-Esteem; viii) Your Child\’s Health; ix) You and Your Family; x) Social Limits Í Behavioral; xi) Family Activities; and xii) Family Cohesion OR The Infant Toddler Quality of Life (ITQOL) tool which is a parent proxy, generic QOL survey for parents/caregivers of children aged 2 months to 5 years\textsuperscript{97, 101}. This 97-item form includes the assessment of a parent\’s perception of their child\’s health and well-being. Domains include: i) Child\’s Discomfort/Pain; (ii)
Child's Temperament and Moods; iii) Child's Behavior Overall; iv) Getting Along with Others; v) Your Child's Health; and vi) Your Child's Impact On You.

2. Pediatric Quality of Life Inventory (PedsQL4.0™) is a parent proxy QOL survey for parents of children 2 years and older. The age-specific tools consist of 23 items and are designed for parents/primary caregivers of children aged 2-4, 5-7, 8-12, and 13-18 years. The domains include an assessment of their child's: Physical, Emotional, Social and School Functioning.

3. PedsQL2.0™ Family Impact Module is a tool that measures the impact of the child's chronic health condition on the caregiver and family. It is a 36-item tool measuring the following domains: Physical, Emotional, Social, and Cognitive Functioning, Communication, Worry, Family Functioning and Daily Activities.

4. Functional Status II-R (short form) is a 14-item parent proxy measure that encompasses the entire pediatric age spectrum and is a generic measurement of health status.

5. Visual Analogue Scales for parent proxy assessment of their child's QOL, overall health, and general pain over the past 3 months as described above.

4.2.1.1 Child Health Questionnaire (CHQ) and Infant Toddler Quality Of Life (ITQOL) Questionnaire

The CHQ and ITQOL were both developed in the United States by Jeanne Landgraf who adopted the World Health Organization (WHO) definition of health for the concept of QOL for both tools. Each tool was developed using a rigorous process for item generation and had been tested for validity and reliability as described in detail below. In order to derive a score for the ITQOL and the CHQ (PF50 and CF87), they require that greater than ½ of the items in each multi-item scale are answered (or ½+1 for an odd number of items). This approach is also used in the SF-36. The scores are recoded and recalibrated by computing the algebraic mean of items for each multi-item scale and then are transformed to a scale ranging from 0 to 100 representing worst to best QOL. The physical activity scale in the ITQOL has an option of "not doing yet" and thus it is suggested, if this option is selected, to exclude these items from the mean scale score. The suggested setting for the CHQ and ITQOL is a mail-out or clinic setting and it was noted that the tools should be completed prior to treatment, medical appointments, and/or procedures to prevent recall bias, according to the CHQ and ITQOL User's Manual.
4.2.1.1.1 The Infant Toddler Quality of Life (ITQOL) Questionnaire

This parent-proxy, generic measure aims to describe QOL in infants and toddlers aged 2 months to 5 years of age with chronic illness and published age-adjusted, healthy normative values were available for comparison. This tool has been used in research for both descriptive and evaluative purposes, however was only used for a descriptive purpose in this study. The ITQOL questionnaire was chosen as it was the only available validated, English language QOL tool for children less than 2 years of age and as young as 2 months of age (a major age group in the GIFT population). The development of the ITQOL began in 1993 but it was not officially released for use until 2006. It has been used in Australian, Canadian, Dutch, and American studies.

**Item Generation and Face Validity:** In the absence of a gold standard for QOL, Landgraf utilized a mathematical, non-targeted approach for item reduction of the multiple-item psychometric scales. The literature suggested that an original pre-release ITQOL contained 103 items but was scaled down to 97 items (9 multi-item scales, 2 global and 2 single item scales with 3-18 items per scale). The ITQOL incorporated results of an extensive literature review, developmental guidelines used by pediatricians, data from the CHQ-PF50 development, and parent feedback during pilot testing. They identified core child health concepts: physical functioning, development, pain, mood, behaviour, general health, family cohesion and parental impact in relation to time and emotions. A psychometric scale approach and likert scales were chosen with a graduated response continuum. There was no mention of the sample size that used for factor analysis and non-targeted item reduction. Typically 10 to 20 respondents are required per item (an absolute minimum of 5) which would result in a sample size of 970 individuals at minimum for development of the items in this tool. The scales appeared to be mutually exclusive which was evident from a higher item-own-scale correlation (discriminative validity). Some studies have demonstrated ceiling effects in certain scales; Raat et al showed ceiling effects (>25% max score) in 3-4 scales, primarily in the general population including physical functioning (79%), bodily pain, parental-emotional impact, and impact on parental-time. The impact on parental-time demonstrated a ceiling effect in both general and chronic illness samples. Easton and Klassen demonstrated a 71% ceiling effect in the physical activity scale as well. There is no summary score for the ITQOL, but rather a
mean score for each scale; summary measures have a theoretical advantage of smaller confidence intervals and elimination or reduction of floor and ceiling effects per scale\textsuperscript{46}. A summary score would facilitate comparison of scores across groups and disease states. Total summation is not possible with the current questionnaire because of the physical activity scale and scales that are specified for ages one year and older. The physical activity scale is related to 10 physical abilities that are specific to the developmental stage. Parents may choose the option ‘not doing yet’ which then results in the item being excluded from the scale score. Scales including ‘your child’s behaviour overall’, ‘getting along with others’ and ‘comparing health from one year ago’ are only intended for children older than one year of age. The completed tool may range from 68 to 97 items preventing total summation.

**Feasibility:** The ITQOL is 97 items in length although it is reported to be easy to complete; in one study only 4\% of responders found the tool difficult or very difficult, 46\% felt neither difficult nor easy and 50\% felt easy or very easy\textsuperscript{59}. The published mean reported completion time was short (14 minutes; range 2-60; SD 7.2)\textsuperscript{59}. The ITQOL response rates in an unsupported setting (mailing) range from 59-83\%\textsuperscript{59, 104, 105, 108} with few missing answers (maximum 6.2\% on 1 item, all others <2.0\%) and few non-unique answers (<0.75\%) \textsuperscript{59}. Published norms in healthy Dutch infants, toddlers and preschoolers as well as Canadian preschoolers were available\textsuperscript{105, 108}. Compared to that available for the CHQ-PF50, the ITQOL lacks robust normative data for comparisons with healthy children\textsuperscript{110} and requires further testing in healthy populations.

Three studies were designed with the primary aim of assessing reliability and validity of the ITQOL which is superior to articles that assess reliability and validity as secondary endpoints\textsuperscript{59, 104, 106}. **Validity:** The ITQOL had been assessed in terms of its concurrent validity (convergent/divergent), discriminative validity, and content validity (face validity). **Concurrent validity** was assessed by correlating scales from the ITQOL with scales of other validated instruments that included both similar and dissimilar scales. Multiple instruments were used for determination of concurrent validity which is a common strategy in view of the lack of a gold standard for validation purposes\textsuperscript{114}. Pearson\textsuperscript{6} and Spearman\textsuperscript{6} correlation values were used to assess this measurement property and were classified as: <0.20 negligible, 0.20-0.34 weak, 0.35-0.50 moderate, and >0.50 strong\textsuperscript{109}. Klassen et al. utilized multiple tools to assess concurrent
validity of the ITQOL with only a vague description of their a priori hypotheses i.e. higher or lower Spearman’s correlations with related and unrelated scales:

- **Child Behavior Checklist (CBCL)** measures social, emotional, and behavioral functioning in children 1 ½ to 5 years of age. This is a 100-item instrument which is summed to a total problem score. This was compared to 55% of the items in the ITQOL scale that measured child behavior or temperament. Getting along, temperament and general behaviour were stated to have correlated strongly (high to moderate) with the CBCL.

- **Short Form 36 (SF-36)** is a popular adult QOL tool with 36 items that measure 8 domains of adult health and was used to validate the parent-impact scales. Overall, parental impact scales correlated moderate to strongly with two of four SF-36 psychosocial scales (energy and social function). The authors concluded that parental impact scales correlated more strongly with SF-36 psychosocial scales than physical scales.

- **Family Assessment Device** is a 53-item measure of family functioning. A comparison of the total score was used to help validate the family cohesion item on the ITQOL. The family cohesion item correlated strongly with the family functioning scale and weakly or moderately with all other scales which provided evidence of convergent and divergent validity.

Raat et al. tested the ITQOL scales with another pediatric QOL scale called the TAPQOL (TNO-AZL Preschool Children Quality of Life Questionnaire). This Dutch questionnaire consisted of 43 items divided over 12 multi-item scales related to physical, social, cognitive and emotional functioning (social, motor and communication were only relevant for children >1 ½ years). A priori hypotheses were set regarding suspected correlation of ITQOL scales and comparative scales and correlations were measured in this study using Pearson’s correlation coefficients. The physical functioning correlated poorly with the motor functioning scale of the TAPQOL with r values of 0.06 for the general population and 0.11 for the respiratory disease population however, the questions differed due to the discrepancy between the target populations e.g. balance, walking up stairs, and running versus rolling over, sitting up, crawling. There were many spurious associations that may have been true correlations between scales or simply a function of multiple comparisons. The TAPQOL was not as well validated as an alternative QOL tool, the PedsQL4.0™, a pediatric parent-proxy QOL questionnaire with modules for children aged 2 to 4 and 5 to 7 years. Unfortunately both scales were inappropriate for
measurement of children’s QOL less than 1 ½ years. The assessment of convergent validity of the ITQOL in the < 1 year age group posed a significant challenge because of the lack of QOL tools available for comparison. **Discriminant validity** is the ability to discriminate between groups of children with poorer expected outcomes and was essential for the use of the ITQOL as a discriminative tool\(^{109}\). The studies used the known-groups method which compared scale scores across groups known or expected to differ in the construct being investigated i.e. chronically ill versus healthy populations\(^{102}\). Klassen used the known-group comparisons with Neonatal Intensive Care Unit (NICU) infants versus a healthy baby sample with the QOL scale scores hypothesized as being lower versus higher, respectively. This was confirmed with significant \(p\) values at the 0.05 level for 9 of 12 scales, excluding bodily pain, mental health and family cohesion\(^{109}\). Easton assessed divergent validity in his clinical sample between three groups of children with dental caries (Caries-free \(n=33\), Chronic Decay \(n=42\), Acute ER visit \(n=50\))\(^{104}\). Mean scores with SD were provided for the three groups and significant differences were seen in relation to only three scales (Discomfort and Pain, Temperament and Moods, and Overall Behavior) as determined a priori\(^{104}\). Raat et al. also assessed discriminative validity by reviewing the number of chronic conditions per child (0 versus \(\geq 2\) conditions) and the number of physician visits in the past year (0 versus \(\geq 4\) visits for the healthy sample and \(\leq 3\) visits versus \(\geq 8\) visits for the chronic population) in both the clinical and healthy populations\(^{59}\). He used two-sided independent sample \(t\) tests and related these differences to previously mentioned criteria for effect sizes. The largest effect size of differences between the known groups was related to the scale on general health (\(k=12\) items)\(^{59}\). There were also moderate to large effect sizes related to bodily pain, temperament and mood; all other scales were associated with small to moderate effect sizes with the exception of the Family Cohesion scale\(^{59}\). This scale was not significantly different between any of the groups and demonstrated a negligible effect size in all comparisons. **Item discriminant validity** was used to establish that correlations of items with other scales were low suggesting that each scale represented separate domains\(^{98}\). Raat et al. defined successful item discriminant validity as an item-own \(r\) value higher than the item-other \(r\) values and found 97% success in the NICU population and 87% in the healthy population. Whereas Klassen et al. specified that the item-own \(r\) value must be significantly higher (\(\geq 2\) standard errors (SE)) than the item-other \(r\) value and found that 12 of 12 scales met this criteria.
Reliability: The internal consistency of the ITQOL was measured in all three studies using Cronbach’s alpha coefficient. As per the limits set by Nunnally, the scale demonstrated adequate internal consistency with Cronbach’s alphas greater than 0.70 on all multi-items scales. Of interest, the ‘physical abilities’ scale (k=10 items) had an alpha coefficient > 0.9 in the three studies that studied reliability. Streiner and Norman suggest that an alpha should not exceed 0.90 as it may suggest a high level of item redundancy. There were two scales within the ITQOL that contained more than 14 items (‘temperament and moods’ and ‘getting along with others’). Streiner and Norman suggest that scales with more than 14 items will result in an alpha of 0.70 or more even if it consists of orthogonal relationships with modest (i.e. 0.30) item-own-scale correlations. Item-scaling testing is only performed on multi-item scales and assesses item correlation within their own scales (Item-own-scale correlation) and other scales (Item-other-scale correlation) by removing the item and then correlating it to each scale. This is best measured by the Pearson Product Moment Correlation (r-value) with an r value >0.20 generally considered a successful correlation. Typically items with an r value less than 0.20 are removed from the scale or rewritten. Most of the items successfully correlated with their own scales; only 60% of the ‘getting along with others’ scale positively correlated 0.40 in both the NICU and healthy baby samples. Raat et al. demonstrated similar results for this scale with an average r score of 0.36 in both the respiratory disease and healthy samples. Test-retest reliability (Reproducibility, Intra-rater reliability) in this setting was defined as a measure of stability of an instrument over time and is often described by an intraclass correlation coefficient (ICC). Raat and Klassen chose a retest interval of 2 weeks. Streiner stated that although the optimal interval between test-retest is unknown, 2 to 14 days was usual. Test-retest reliability was measured on the individual level by an ICC where a value 0.70 was considered adequate and 0.5-0.7 moderate. Families in the Klassen study had agreed in advance to do the retest and all scales were found to have an adequate ICC. Raat et al. investigated test-retest in a random subgroup of 229 individuals with an equal number from the clinical and healthy samples. Upon individual assessment ICC values were adequate in only 4 of 12 scales while the majority were moderate. Low to moderate ICC values may indicate poor stability of the ITQOL over time or simply reflect a true change of the measured scales and/or construct.
Normative Comparison Sample: The ITQOL pre-published comparative sample for ages 2 months to 5 years was a random Dutch sample including 410 healthy children that were a part of six municipalities followed by a Well-Child Care regional provider\textsuperscript{59}. Ninety-seven percent of the respondents were mothers, the majority born in the Netherlands with a secondary school education. The gender of the children was evenly distributed and the mean age was 24 months with a range of 3 to 46 months of age. The normative data was available for all multi-item scales on the ITQOL excluding the global items for health and behaviour.

4.2.1.1.2 Child Health Questionnaire (CHQ) Parent and Child Forms

The CHQ, also developed by Landgraf, was the original tool that underwent significant stages of development and psychometric testing beginning in the early 1990's. The CHQ measures both physical and psychological aspects in keeping with the WHO definition of health. The CHQ is available in three versions and are labeled according the name of the tool, respondent, and number of items\textsuperscript{97}: the CHQ-PF50 (Parent Form 50 items), the CHQ-PF28 (Parent Form 28 items), and the CHQ-CF87 (Child Form 87 items). The most frequently applied version of the CHQ questionnaires is the CHQ-P50\textsuperscript{40}. The CHQ-PF28 will not be discussed as it was not utilized in this study. The CHQ has extensive published normal values available for reference and interpretation of research. In 2001, the CHQ was cross-culturally validated using forward-backward translations in 32 countries\textsuperscript{117} and since has been translated into over 60 different languages\textsuperscript{97}. Waters et al. pilot tested the CHQ-CF87 in an Australian population of adolescents and recommended the elimination of 7 items, modification of some activities to match their sample and a higher age range (12-18 years) although the data and justification for this was not presented\textsuperscript{118}. Nonetheless as a result of their studies, a new international reference population was established (n=2361)\textsuperscript{118}.

Item Generation: Landgraf intended to develop a general functional status and well-being instrument for ages 5 and older however it was a difficult task to produce one form given that developmental stages are very different in this age range\textsuperscript{97}. It was important to note that the initial goal of these tools was not to develop a QOL tool; however the tool has been used to assess the construct of QOL for 20 years. Landgraf started with a tripartite measurement model that included status, disability and personal evaluation with a minimum of a physical and
Landgraf felt that a deficit in either aspect would impact a child's ability to participate within their social role such as attending school, playing with friends, and developing relationships. In order to develop this tool, they reviewed existing tools and projects, reviewed previous experience in tool development and consulted experts in child development. They subsequently developed core concepts and ranked them according to agreement. It was unclear whether children or parents were involved at this stage of item generation which would have been beneficial. In 1990, they tested the first full length tools; CHQ-PF98 and CHQ-CF87. The PF50 was designed using regression techniques and item scaling analysis. In general, most of the questions are based on an assessment of the last 4 weeks except for Change in Health which relates to the past year and Family Cohesion and General Health on the PF50 which have no time reference.

Factor analysis using the principal components method was performed for the PF50 and consistently pointed to two factors which accounted for 59.2% of the total variance, satisfied eigen values, the scree test, five percent rule and the common factor test. The Physical Summary Scale was most highly correlated with Physical Functioning, Role-Social/Physical, Bodily Pain and Perceptions of General Health. The scales mostly correlated with the Psychosocial Summary Scale included Role-Emotional/Behavioral, Self-Esteem, Mental Health, and Behavioural. Parental Impact Emotions and Parental-Impact Time both correlated with the Psychosocial scale but had secondary correlations with Physical. The psychosocial and physical summary scales are calculated only with the CHQ-PF50. An overall QOL summary score cannot be calculated for the CHQ-PF50 and the CHQ-CF87 survey and the reasons supporting this were not discussed in the manual.

**Feasibility:** Flesch-Kincaid readability estimates were performed for both tools. It was determined that 80% of individuals reading at a grade 3.5 level understood the CHQ-PF50 and 85% of individuals reading at a grade 2.5 level understood the CHQ-CF87. A sample of CHQ-PF50 tools were sent out to a normal, healthy population (n=391) and a clinical sample (total sample size unknown). Ninety-one percent of the total normative sample returned completed questionnaires with a range of 85-95% completion within the subgroups. Upon examination of the subgroups, it was determined that questions were less likely to be complete for minorities (85%) and those with less than a college education (89%). Subsequently, there was a review of
the particular scales and one item in the Family Activities scale was reworded. The clinical sample completion rate ranged from 74-94% however the data for the PF50 was within the original PF98 and also included many disease specific modules (i.e. battery assessment)\textsuperscript{97}. The results tended to improve with less questions asked; for example, those asked to respond to 180-210 questions resulted in a range of 74-84\% and those with 108 items had 93\% completeness\textsuperscript{97}. Unfortunately the 2008 edition of the CHQ manual does not present convergent/divergent validity results and floor and ceiling effects. Waters et al. in the Australian version of the tool found significant ceiling effects in three of the same four scales of the CF87 that Raat et al. revealed in a psychometric analysis of the Dutch version (physical functioning, role functioning, emotional/behavioral and physical)\textsuperscript{118,119}, although the ceiling effects in the Raat study were significantly higher. Raat et al. in a later study in 2007 investigating paper versus online administration in adolescents found consistent results in ceiling effects\textsuperscript{120}. This would limit the use of detecting health changes in healthy adolescent populations however it was unclear what the impact would be on a clinical sample.

In terms of the CHQ-CF87, the assessment of data completeness was done in a predominantly African American population of children from 10-15 years of age and in three clinical samples of children with Attention Deficit Hyperactivity Disorder (ADHD), Cystic Fibrosis (CF) and ESRD. The tool was provided in addition to three demographic questions and a checklist of 9 symptoms and was completed in a classroom or clinic setting\textsuperscript{97}. The range of completeness in the school setting was from 53-74\% with a mean of 63\% and was lowest in children from 10-12 years of age (53-65\%)\textsuperscript{97}. Overall, 29\% of the surveys with missing data were missing only 3 items or less. In the clinical setting, 63-77\% of the surveys were complete with the lowest in the CF group (63\%)\textsuperscript{97}. In both groups, some scales including behaviour and mental health were missing answers related to subjective responses or the negative options (e.g. lies or cheats).

**Validity:** Validity was discussed in the CHQ manual in a variety of ways that related to the development of the tool, such as comparing to other child and adolescent scales, discussion of the integrity of items and rigorous translation, factor analysis for content validity and the development of norms for reference and interpretation \textsuperscript{97}. Although these are important aspects of the tool they do not specifically demonstrate validity.
**CHQ-PF50 – Discriminant Validity** of the PF50 was assessed using the known groups method from their large data set of normal children and 5 of the 7 clinical samples. They hypothesized that school children would have better scores than the clinical sample, that debilitating conditions would have lower scores than others (e.g. epilepsy), that the physical functioning scores should be the lowest for children with Juvenile Rheumatoid Arthritis (JRA) and that lower scores should be seen in the psychosocial summary scales in children with ADHD. These hypotheses were tested statistically by comparing mean differences with a paired-t test statistic for paired comparisons. Each of the 4 hypotheses was confirmed overall and many differences seen between groups could also be explained clinically. In addition, the authors reported an F statistic to demonstrate discriminant validity of scales relative to one another within the groups (normal and clinical) and found that these values were highly significant with a p-value less than 0.001 for each scale. **Concurrent validity** has been assessed comparing the CHQ-PF50 to the HUI (Health Utilities Index), a pediatric health status measure in a population of childhood cancer survivors. A priori hypotheses were stated regarding scales that would correlate and the degree of effect size and some were found to be stronger than originally expected. Strong correlations were present with pain and mental health and emotions, moderate correlations with physical functioning aspects and general health and global utility scores. In contrast, Raat et al. found in a population of 367 Dutch children (aged 5-13 years) that the predicted associations were quite weak between the same two tools however, each scale correlated more strongly with the predicted scale than with the other scales. **Construct validity** was assessed in children and adolescents with ADHD (n=761) and found that as ADHD-related symptoms increased in severity, the mean psychosocial summary score of the CHQ-PF50 decreased.

**CHQ-CF87 – Discriminant Validity** was measured in one of the original articles by Jeanne Landgraf where a school sample was compared with 2 clinical samples (ADHD and ESRD) using item-own-scale correlation and item-other-scale correlations. A success was counted when an item correlated ≥ 2 standard errors with its hypothesized scale. All 10 scales in the school sample demonstrated very high success (between 92-100%), as well as 8 of 9 scales in the ADHD sample and 6 of 9 scales in the ESRD sample. The lowest were the general health and the behaviour scores in the ESRD sample, 62 and 79% respectively however this sample only had 19 children. Raat et al. investigated **Construct Validity** in the Dutch adolescent population.
and found that all mean scale scores reduced with an increase in reported conditions (0 versus 1-2 versus $\geq 3$).

**Reliability:** The CHQ has been assessed in terms of **Internal Consistency** using Cronbach\(\alpha\) alpha coefficient as an average correlation between all sets. A minimum reliability standard of $\geq 0.70$ was felt to be adequate to claim that the tool could be used for group level analyses and 0.9 for individual analysis, however an alpha greater than 0.90 may suggest redundancy\(^{46}\). These reliability results were summarized in the CHQ manual and were from numerous studies with differing methodologies, many of which were unpublished. The analysis was designed to review 16 subgroups of children, both boys and girls, with varying ages (5-7, 8-10, 11-12, 13-15, 16-18yrs), both maternal and paternal respondents, and varying ethnicity, education level and work status\(^{97}\).

**PF50 – The internal consistency** Cronbach\(\alpha\) alpha estimates, including ranges and medians, were determined in both a normative sample (n=379) and clinical samples including asthma, ADHD, CF, epilepsy, JRA, and psychiatric (n=3415; 72% of this sample was a large HMO sample of patients with asthma)\(^{97}\). Ninety-one percent of the coefficients from the normative sample were $\geq 0.70$ (ranging 0.6 to 0.94). The lowest coefficients ranged from the 8-10 year and 11-12 year samples, with results $\geq 0.70$ in 73% and 70% of these samples respectively). However upon review of the ranges, some coefficient ranges were as low as 0.46 and as high as 0.96\(^{97}\). When the subgroups were further examined in terms of gender, both were considered statistically adequate with a median coefficient of 0.77 for girls and 0.92 for boys. A priori hypotheses concerning subgroups were established. The following statements were anticipated and confirmed with reliability estimates $\geq 0.70$\(^{97}\): (a) higher estimates in parents reporting some college education versus those with high school education or less (91% versus 82%), and (b) nonworking versus working parents (90% versus 82%). Some results were unexpected in that (a) higher estimates were found in non-Caucasian versus Caucasian parents (91% versus 82%), and (b) dads versus moms (91% versus 80%)\(^{97}\). There was significant disparity in the sample sizes within some of the subgroups and thus results were interpreted with caution. On a scale level, reliability estimates were not determined in all clinical samples for each scale (not all studies included each scale). Greater than 86% of the samples had Cronbach\(\alpha\) alpha\(\geq 0.70\) in 6 of 10 clinical samples, with lower percentages found in 2 asthma groups, ADHD and JRA (as
The majority of estimates for Role Emotional/Behavioral, Self-Esteem and Behaviour were greater than 0.80. The majority of results for Physical Functioning, Bodily Pain, and Limitations in Family Activities were greater than 0.90. The authors conclude that this is appropriate for individual level analysis however this may also demonstrate redundancy in these scales. The lowest overall median coefficient was consistently for General Health Perceptions (0.68 median, range 0.46 to 0.80) which the authors defended under the premise that it was intended to provide heterogeneous results and to avoid bias, the question was worded with both favourable and unfavourable options resulting in balanced responses. This was consistent with a study of adolescents with Type 1 diabetes where the General Health scale on the PF50 had a reliability coefficient of 0.63 and all other scales ranged from 0.70 to 0.95.

Test-retest reliability was only done on the Dutch version of the PF50 in 47 subjects assessing ICCs with a level of significance rather than classifying the values i.e. poor (<0.40), moderate (0.40-0.75), good (0.75-0.90), excellent (>0.90). The ICCs ranged from 0.31 to 0.84, with most values falling between 0.40 and 0.75 i.e. moderate agreement.

Internal consistency was measured in a school-based sample (n=263) and three clinical groups (ADHD (n=55), CF (n=30) and ESRD (n=20)). Across all 4 samples, the median correlation coefficients were greater than 0.70 with ranges between 0.73 and 0.97. It was stated, but not presented in the CHQ manual that each scale met the minimum median correlation coefficient of 0.70 except for the General Health Perceptions, similar to the PF50. The median coefficient was greater than 0.90 in the Physical Functioning scale in 2 samples: ADHD and CF as well as in the Role/Social-Physical scale in the CF population. Overall, however most fell between 0.70 and 0.90. In addition, it is important to note that the Family Activities scale was only administered in the normative school population and thus results were not available for the clinical sample. Wake et al. found reliability coefficients from 0.73 to 0.97 in adolescent patients with Type 1 Diabetes. Test-retest reliability was tested in the translated Dutch version of the CF87 but not in the American version. The questionnaire was given to 71 schoolchildren 2 weeks after the initial administration and was found to have significant differences in test scores with small effect sizes (0.25-0.40). All ICC values were significant at p value <0.01 except for Physical functioning and Role functioning physical. Further testing is required to ensure adequate test-retest reliability.
Summary Scales (Physical/Psychosocial) – Reliability of these two summary scales was assessed in both the PF50 and PF28 utilizing internal consistency reliability of each scale and their covariances. Only the results of the PF50 will be discussed here and will be based on the normative sample of 380 proxies and 7 of the 10 clinical samples. Group level and individual level testing was examined in the normative and clinical samples and found to be reliable in both scales at the group level (Ô 0.70), all groups for the psychosocial summary scale at the individual level (0.90) and 14 of 16 subgroups for the physical summary scale individual level testing (excluding girls I 0.86 and non-working parents I 0.88).

Normative Data: The children for the normative data for the PF50 came from a sample of 391 children from the United States who were surveyed as part of the 1994 National Survey of Functional Health Status (NSFHS). The PF50 was embedded in a larger survey and was completed by proxies of children (55% male) with a mean age of 12 years. The majority of the proxy respondents were female (65%), had a minimum of college experience (60%), and were Caucasian (83%). The respondents of the survey were compared to the general US population using the National Center for Health Statistics and were felt to be representative. The normative data for the CF87 was from a 1997 publication by the author of the CHQ, J.M. Landgraf, which included 232 children from 3 cultural groups (African-American, Spanish-American and Caucasian) who attended middle school in 1995 in a moderately large suburb in the northeastern United States. Fifty-eight percent of the sample was female and the mean age was 13 ± 1.1 years, ranging from 10 to 15 years.

In summary, there has been extensive testing in internal consistency reliability and concurrent and discriminative validity testing for the CHQ-CF87, CHQ-PF50 and the ITQOL. Further testing regarding sensibility and test-retest reliability would be beneficial for all tools. Consideration of an overall summary score for QOL would be useful for comparisons across populations and interpretation of results.

4.2.1.2 The Functional Status II(R) Short Form

The Functional Status II(R) short form (FSII(R)) is a 14 item proxy measurement for the entire pediatric age spectrum and was developed as a measurement of health status. Specifically it was stated to be designed for assessment of disease impact on physical, psychological and social
functioning for kids with chronic disease. The scale was described by Klassen et al. to be attempting to describe whether a function of health was related to the child’s health problem or simply an unrelated issue. It has been used in many pediatric studies including cancer, congenital diaphragmatic hernia, and cardiac transplantation. An article published in 1990 by the author of the FSII(R) clearly detailed the development and psychometric testing of the second version of the Functional Status measurement tool.

**Tool Development & Item Generation:** The Functional Status I (FSI) was developed in 1978 and intended to measure individual child health status and to distinguish multiple populations. The FSI was modeled after the adult measure of functional status, the SIP (Sickness Impact Profile), and viewed behaviour as the final common pathway of health. Stein et al. defined a healthy child as ‘one who exhibits age-appropriate physical, psychological, intellectual, and social behaviors’. The scale items were stated to be based on a thorough literature assessment, interviews with mothers and experienced health care providers (sample sizes not provided), and clinical experience of the authors. It was unclear how many items were in the original item pool but they did state that the item pool was reviewed by an expert panel for content, clarity and relevance to the construct ‘dysfunction due to illness’. Their goal was to provide a measure that was sensitive enough to detect the range between healthy children and those with severe disability. The FSI was divided into four age categories: infants, toddlers, preschoolers and school age children. In 1990, Stein et al. set out to refine the FSI via new data acquired from a sample of healthy and chronically ill children. They decided to improve clarity of certain items (i.e. improve face validity), increase the age span of the questions to the largest possible range, and to test the revised tool on a sample of children for assessment of construct and concurrent validity and the ‘applicability’ of the measure to variable populations. Initially 53 items were developed and 35 were unchanged from the FSI, 5 items from the original were deleted and 3 new items were added. The tool was administered in an interview format by a trained interviewer with mothers or the primary caregiver of the hospitalized child in English or Spanish. Typically 10 to 20 respondents are required per item (an absolute minimum of 5) which would result in an appropriate sample size of 530-1060 individuals for this tool. This was achieved by testing the tool on 732 children from ages 2 weeks to 16 years in a Pediatric Department in New York State. These children ranged from those with significant chronic
illness, to those with ongoing healthy concerns being followed regularly, to those seen for healthy child visits.

Principal component analysis to assess a one-factor solution and factor analysis with varimax rotation were used with the criteria of retaining an item with a minimum factor loading of 0.4\textsuperscript{103}. The primary analysis was done with the chronically ill children (n = 242) and then a secondary analysis combined both chronically ill and well children. The final tools resulted in a 43-item long form and a 14-item short form. The single shortened form has a range of core items that are applicable across the age ranges and was stated to have excellent psychometric properties although the development of this form and the steps taken were not presented, nor was there a reference available\textsuperscript{103}. The FSII(R) was designed such that each item involved a pair of questions. The first portion of the question is regarding the child’s behavior over the past 2 weeks and the second asks the respondent to consider how much of this was attributable to the child’s illness. e.g. Here are some statements that mothers have made to describe their children. Thinking about your child during the last two weeks, did (he/she)É .

Eat Well:

<table>
<thead>
<tr>
<th></th>
<th>Never or Rarely</th>
<th>Some of the Time</th>
<th>Almost Always</th>
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<tbody>
<tr>
<td>Was this due to illness?</td>
<td>Fully</td>
<td>Partly</td>
<td>Not at all</td>
</tr>
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</table>

**Scoring:** The FSII(R) is scored as the percentage of the total points achieved to the total possible points of the entire scale. If a respondent indicates that the child’s behaviour was not at all due to illness, then the response is changed to a higher score. This is done to ensure that a lower score is attributed to illness. There are no cutoff values for this tool or ranges for classification rather, it is used for comparative purposes and is dependent on the chosen group for comparison\textsuperscript{103}.

**Validity:** **Discriminant validity** was assessed by comparing the scores of the ill versus the well children with the expectation that the well children would have higher mean scores. The mean scores +/- SD on the 14-item scale for all ages were 86.8 +/- 15.7 for ill children and 96.1 +/- 8.2 for the well children\textsuperscript{103}. Stein et al. felt that this proved discriminant validity and also supported previous findings that despite different values, chronically physical disordered children function as well as other children. **Concurrent validity** was assessed by correlating FSII(R) scores with measures of morbidity status (e.g. days in hospital in past 6 months, days in bed in past 2 weeks,
hospitalizations in past 6 months and days away from school in past 2 weeks). The data was presented in correlations but analyzed with levels of statistical significance rather than strength of association which can be deceiving especially in view of a large sample size. Despite being statistically significant, most of the correlations were weak in the chronically ill child group except for the older children's days in bed and absences from school over the past 2 weeks which had moderate to strong correlations, using Cohen's effect size guidelines. In contrast, the older children who were well did show moderate to strong associations in most indicators. The 14-item measure scored very low correlations in the past 6 months indicators (chronic r = -0.13 to -0.24; well r = -0.13 to -0.10) and moderate to high correlations in the past 2 weeks indicators (chronic r = -0.37 to -0.47; well r = -0.28 to -0.58). The FS II(R) asks parents to assess their children over the past 2 weeks and the time frame for two of the traditional health indicators chosen to measure concurrent validity assessed over 6 months which limits its value for comparison. Construct validity was assessed by examining three specific questions from the tool i.e. child looks different, as physically active as others, and as able to learn as others and determining whether there were significant differences in the mean scores within the response options of these groups. They performed an ANOVA using these categorical variables and all F statistics were significant at the p value of <0.01 to <0.001.

Reliability: Cronbach's alpha was appropriately applied to determine internal consistency and all alphas were greater than 0.80. As previously mentioned, if alpha is greater than or equal to 0.90, Streiner and Norman suggested this may be due to a high level of item redundancy. Some might argue that the 43-item scale may have been shortened. However, the short form 14-item was also shown to have high Cronbach's alphas in the range of 0.83-0.91 although less so than the long form. A multi-centre study of pediatric oncology patients had greater than 400 parents complete the FSII(R) and demonstrated an acceptable Cronbach's alpha of 0.84. Due to the large age range of administration, an analysis using various age groups was completed with consistent results. Test-retest reliability had not been measured to our knowledge.

Normative Sample for Comparison: The published normative data was determined using two samples of children, classified as chronically ill (n=456) or well (n=276) and was conducted in November 1990 in New York State. Both samples were recruited from a pediatric department that included children with significant chronic illness admitted to a tertiary care centre,
subspecialty clinics or those attending routine health care clinics with no physical health conditions\textsuperscript{103}. The ages ranged from 2 weeks to 16 years of age and included both urban poor and middle class families.

The tool that was used for this study, FSII(R) short form, had minimal data available to assess reliability (other than internal consistency), validity and tool development. Further studies would provide a better understanding of the psychometric properties of this version of the tool.

4.2.1.3 Visual Analogue Scale

The visual analogue scale (VAS) was defined by Wewers and Lowe as a straight line, 10cm (100mm) in length, and has an anchor on each end labeled as the extreme of a sensation, feeling or response\textsuperscript{99}. The subject responds with a mark on the line which signifies their perception of the phenomenon\textsuperscript{99}. The VAS was used in the current study as a single dimensional assessment of quality of life, pain and overall health (See Appendix I). Although it is difficult to assess specific VAS scores, they do give a broad, subjective perception of each tested concept\textsuperscript{47}. Multiple lengths of the VAS have been tested and it was found that the 10cm length had the smallest measurement error in comparison to 5cm and 20cm lines\textsuperscript{130}. A 10cm VAS was used in this study for proxy respondents of children 2 months to 18 years and self-completed by children ages 8 years and up. Visual analogue scales have been used in children as young as 7 years old\textsuperscript{131} and children 8 years and older have demonstrated the ability to respond to a question with a 4-week recall period with accuracy\textsuperscript{132}. In this study, there were three VAS scales asking the rater to assess, over the past 3 months, their (1) overall QOL, (2) overall health and (3) overall pain. Zero indicated the worst possible score and 10cm indicated the best possible score for all constructs. The timeline in this study (3 months) was greater than that assessed in the above mentioned study (4 weeks) and was considered in the interpretation of the responses of the young children. The VAS has been used for the measurement of multiple concepts including mood, anxiety, cravings, quality of sleep, attitudes, pain, quality of life, nausea, vomiting, dyspnea, and functional abilities. An article by Wewers and Lowe in 1990 summarized the psychometric evaluation of the single-item VAS, primarily focusing on the pain and mood literature\textsuperscript{99}. 
Feasibility: The VAS is simple to administer, easy and cost-effective however it is difficult for some, especially young children, to understand the representation of an intangible concept as a line and each individual will interpret the spectrum between the two ends differently99.

Validity: Construct validity was measured in pain studies in the early 1980’s and found the VAS to be able to discern changes in pain133,134. In addition, the VAS measuring QOL was studied in four groups of adult oncology patients receiving different treatment regimens where an expected level of QOL was determined and these hypothesized differences were confirmed135. Stinson et al. reported moderate to strong correlations (r= 0.63–0.90) with many other pain measures in children136-139. Discriminant validity was examined in pain and anxiety. Wewers et al. described two studies that showed a difference in the VAS with two different levels of pain however these differences were non-significant. One unpublished study was described by Wewers et al. as a multitrait-multimethod approach examining discriminant and convergent validity between pain and anxiety scales140. The discriminant correlation between the two VAS, pain and anxiety, was r= 0.49, only slightly lower than the convergent correlation between two pain measures and two anxiety measures (r= 0.53 and r= 0.67, consecutively) 140. However, an argument could be made that pain and anxiety were not appropriate choices for measuring divergent validity which requires no or opposing relationships between the two factors.

Criterion-related validity was assessed by comparing the VAS measure to an existing ‘gold standard’. One of the major challenges in the assessment of validity in health care is the lack of appropriate gold standards for comparison141. This was allegedly demonstrated in pain, mood and cravings by comparing to other published, validated scales99. However it should more appropriately be stated as evidence of concurrent validity.

Reliability: Reliability testing of the VAS was difficult to accomplish in that it is a single construct and is often used to measure dynamic concepts such as pain and mood99. The most commonly used test of reliability was test-retest reliability. Varying periods of time were selected including a few minutes for analgesia in dental work133, 5 minutes and 24 hours for remembering distant pain142, and up to 2 days. However, for this psychometric property, these time periods are only suitable in constructs that are not expected to change99. The correlations from these studies ranged from 0.95 to 0.99, demonstrating strong test-retest reliability. Another study attempted to score mood repeatedly however this was also a dynamic construct and
therefore correlations were between 0.41 to 0.93\(^9\). Stinson et al. published a review article in 2006 to examine the use of self-report pain intensity measures for use in clinical trials\(^{143}\). Upon review of the VAS, Stinson described a 1985 abstract by McGrath et al. that found a moderate to strong positive median correlation (r=0.70) in 5-6 year olds where test-retest assessed pain over a 2 week interval. In the early 1980\(^\text{nd}\)s, Dixon and Bird looked at the reproducibility along the length of a vertical 10cm (100mm) VAS in normal subjects and found that the extremes were the easiest to reproduce but the midpoint of the line +/- 2cm was the most difficult\(^{144}\). Other reliability issues that have been reviewed included the position of the line, allowing subjects to view their previous ratings, and issues related to visual and psychomotor abilities of individuals. The most important aspect of direction was maintaining the same direction of the line if you were doing multiple measurements as there was some evidence of lower ratings on the horizontal line\(^{145}\). The availability of previous ratings for reassessment of the same phenomena remains controversial\(^9\).

The VAS is a feasible tool that is useful in determining an overall assessment of a particular construct. Reliability is difficult to assess in these one-dimensional assessment and has mainly focused on the presentation of the tool. Test-retest reliability and concurrent validity have been tested. The majority of the testing in its psychometric properties has been conducted in terms of pain and mood with one adult study on QOL. There was no evidence related to overall health.

### 4.2.1.4 PedsQL4.0\(^\text{TM}\) (Pediatric Quality of Life Inventory) Parent and Self Reports

The PedsQL4.0\(^\text{TM}\) was developed by James Varni, PhD as a generic measure of health-related QOL and was first published in 1999\(^{40}\). The current version of the PedsQL4.0\(^\text{TM}\) was developed over 15 years and expanded on earlier versions of the PedsQL\(^\text{TM}\) by adding additional constructs, increasing the age ranges and improving sensitivity on the scale range with the ongoing goal of measuring the core dimensions of health as defined by the WHO: physical, social and psychological aspects\(^9\). Varni et al. suggested the use of the PedsQL4.0\(^\text{TM}\) generic measure in conjunction with the PedsQL\(^\text{TM}\) disease-specific modules which were available for arthritis, asthma, brain tumour, cancer, cardiac disease, cerebral palsy, diabetes, rheumatology and transplant\(^{146}\). The combination of these two types of measures (disease-specific and generic) may have provided a more complete measure of QOL with increased sensitivity for the issues
related to chronic health diseases\textsuperscript{98}. However, this was not available for the patient populations in this study. The PedsQL4.0\textsuperscript{TM} has undergone translations however it has not undergone the same full cross-cultural validation process as that of the CHQ\textsuperscript{40}.

**Item Generation and Tool Development:** The PedsQL\textsuperscript{TM} conceptual model was based on symptoms and the impact of cancer on pediatric patients as it was thought that those experiences would overlap with many other chronic diseases\textsuperscript{60}. These children (n=291) were aged 8-18 years, at variable stages of treatment, disease staging and progression with a mean middle-class socioeconomic status. The items in the PedsQL\textsuperscript{TM} were generated and refined over a 5 year period through 6 cycles of extensive literature reviews, multiple focus groups, cognitive interviews with patients and families, discussion with health care professionals, multiple administrations and field testing\textsuperscript{98}. The items were first derived for the child-self report scales and then the proxy-report scales were developed to directly parallel the child items\textsuperscript{147}. Initially, the tools contained 84-87 items and after undergoing field and correlation testing, it was reduced to 45 items\textsuperscript{60}. Rebok et al. demonstrated that school-aged children from the age of 8 years old understand the range of and rate themselves on 5- and 7- point scales however younger kids tend to use the extreme options\textsuperscript{132}. Consistent with this literature, Varni et al. used a 5 point response scale for all parent proxy reports and all child self-reports from age 8 years and up (0 = never a problem, 1 = almost never a problem, 2 = sometimes a problem, 3 = often a problem, 4 = almost always a problem). A simplified 3 point scale was used for the young child (5-7 years) self report (0=not at all a problem, 2=sometimes a problem, 4=a lot of a problem) and each response was anchored on a continuum with a happy to sad face scale\textsuperscript{98}.

**Scoring:** Scoring of the PedsQL4.0\textsuperscript{TM} was similar to the CHQ/ITQOL by reverse-scoring each item and linearly transforming responses to a score from 0 to 100. However, the scoring was more straight-forward in that the response options did not change and all items were reverse-scored as opposed to forward and reverse scoring on the same scale, as in the CHQ. The scale scores and total scores were recoded and recalibrated by computing the algebraic mean of items for each multi-item scale and then were transformed to a scale ranging from 0 to 100 representing worst to best QOL\textsuperscript{146}. If greater than 50% of the items were missing then it was suggested not to total scale scores\textsuperscript{146}. Summary scale scores are calculated as the mean of the responses for Physical, Emotional, Social and School Functioning. The Physical Health
Summary score is equivalent to the Physical Functioning scale. The Psychosocial Summary Score is calculated as the average of the Emotional, Social and School Functioning. The Total QOL Score is calculated based on the average of all completed items.  

**Feasibility:** The first large study to assess the psychometric properties for the PedsQL4.0™ included children aged 5 to 18 years and parents of children aged 2 to 18 years with 1677 participants in total (in 915 children both child self-report and parent proxy were available). These participants were recruited from three sites: well child pediatrician appointments (n=98, 5.9%), specialty hospital clinics: orthopedic (n=127, 7.6%), cardiology (n=156, 9.3%), rheumatology (n=95, 5.7%), diabetes (n=49, 2.9%), and children seen as inpatients and outpatients at the Children’s Hospital and Health Center in San Diego or its affiliated outpatient community clinics (n=1141, 68%). Administration was achieved by two modes, 26.2% in person and 73.2% by telephone and were completed in two languages, English (79.2%) and Spanish (20.8%) using a heterogeneous ethnic sample including white non-Hispanic, black non-Hispanic, Hispanic, Asian, American Indian or Alaskan Native. Information on non-participants was not collected for this study. No differences were found comparing the mode of administration and language in terms of missing data, floor and ceiling effects and scale internal consistency. Feasibility was determined by the distribution of item responses and the percentage of items that were left without a response. The item responses tended to skew towards a higher QOL however did show a full range of responses. The percentage of items that were missing responses was 1.54% for self-report and 1.95% for parent proxy (in a separate study of parent proxy, the result was consistent at 2.1%). The scale-level analysis demonstrated no basement effects (only 2.3% scored the minimum) and ceiling effects existed minimally in the healthy population for the Total QOL Score (self 7.2%, parent proxy 10.3%) and moderately in the same population for the Social Functioning subscale (74.1% for self and 58.1% for proxy report). The healthy population had more ceiling effects than the chronically ill sample on all subscales.

**Validity:** Concurrent Validity was measured during the development of this tool and additionally when the tool was tested further for psychometric properties. During development, it was successfully compared to measurements of emotional distress, social support, perceived competences and functioning with the Children’s Depression Inventory (CDI), the State-Trait Anxiety Inventory for Children (STAIC), the Social Support Scale for Children and Adolescents.
(SSSC), the Self-Perception Profile for Children (SPPC) and Adolescents (SPPA) and the Child Behavior Checklist (CBCL) demonstrating moderate correlations with hypothesized scales. Once the tool was finalized, it was tested for **construct validity** using the known-groups method, comparison with currently published scales, and correlations with various measures of morbidity and illness burden. The known-groups method hypothesized that healthy children would have higher QOL scores than children with acute or chronic health conditions using ANOVA while examining confounding for gender, ethnicity, age, and health status. It was found that there was cultural disparity within the chronically ill group versus the healthy group, however tests of interaction were conducted and found not to be an issue due to large sample size and small eta-squared values. Therefore, the initial hypotheses were confirmed and the scores were higher in healthier children. This was also shown in a 2007 study by Varni et al. looking only at parent proxy reports with statistically significant higher mean scores (i.e. better QOL) in the healthy children with medium to large effect sizes. The original methodological study consisting of pediatric cancer survivors found significant differences between groups with cancer on treatment versus off treatment (p<0.0004), with lower versus higher QOL consecutively. In 2002 this was measured cross-sectionally in a pediatric cardiac population (n=115) whose disease severity was measured by a system developed by the New York Heart Association. These four classes included: Class I to IV with worsening symptoms and functional limitations as the class level increased. The PedsQL™ generic core scales demonstrated a decreasing QOL with increasing class of cardiac disease in both the self-report and parent-proxy report. Using ANOVA, significant differences at a p-value <0.05, were determined in the total scores between Class III/IV and Class I, Class II and the healthy population in both types of reports. Construct validity was also assessed by looking at absences from school and work for parents, medical care required in the past month, and impact of child’s health on the parent’s functioning by determining a priori hypotheses and associations with higher mean scores on the PedsQL™. Most correlations were small relative to the child report, except there was a medium correlation with impacting the parent’s ability to function. Parents demonstrated medium to large correlations with the same aspect on the parent proxy forms. On a scale-level, a multitrait-multimethod strategy was used to assess **convergent and divergent validity** and confirmed both measurement attributes.
Reliability: Item-Scaling Testing was completed using multitrait scaling analyses to approximate the item scaling success. A success was defined as the number of times an item correlated with its hypothesized scale rather than another scale by ≥2 standard errors and was found to be successful between 87 to 100% for both reports. Item-Scaling Testing is only performed on multi-item scales and assesses item correlation within their own scales (Item-own-scale correlation) and other scales (Item-other-scale correlation) by removing the item and then correlating it to each scale. A Pearson correlation coefficient ≥0.20 is generally considered a successful correlation however adequate item-own-scale correlation was defined as a minimum of 0.40 in these studies. It was stated that 19 of 23 items in the self-report and all items in the proxy-report met or exceeded the 0.40 standard however the data was not presented in the paper. Internal consistency reliability of the scale used Cronbach’s alpha and demonstrated values greater than 0.70 in all scales except for the School Functioning scale in the Self-Report (r=0.68). The total QOL scores for child and parent-proxy were 0.93 which suggests that it is appropriate for individual analysis, however possibly a high level of item redundancy.

Normative Data Comparison: The normative data was published in a 2007 article by Varni et al. which compiled much of the published PedsQL4.0 data and compared healthy children to those with chronic disease. Varni et al. was successful in recruiting very large numbers of study participants to develop an impressive database over 7 years of published normative comparisons. Approximately 9430 parent proxies of healthy children were recruited via an insurance study and also during the initial field testing of the PedsQL4.0. These children were attending well child checks and reported no chronic conditions prior to proxy completion of the QOL questionnaires. The chronically ill sample was recruited from 10 physician diagnosed disease clusters and 33 disease categories from general pediatric clinics, specialty clinics and hospital admissions. The published norms for the Self Report of the PedsQL4.0 were recruited in the same study and via same methodology as the aforementioned proxy report. Varni et al. recruited 5500 healthy children to complete self reports in order to publish the current normative data results.

There was extensive literature regarding the validity and reliability of the PedsQL4.0 generic scale scores. The development of the tool was outlined although more detail could have been
provided in terms of sensibility and item generation. Another benefit of this tool included the large database of normative values for comparison.

4.2.1.5 PedsQL™ Family Impact Module

The PedsQL™ Family Impact Module was also developed by Varni et al. as a complementary or stand-alone parent proxy multidimensional instrument that would measure the impact of pediatric chronic health conditions on parent and family functioning. This 36-item parent proxy instrument includes 6 subscales that measure the parents’ self-reported functioning: Physical Functioning (6 items), Emotional Functioning (5 items), Social Functioning (4 items), Cognitive Functioning (5 items), Communication (3 items) and Worry (5 items). There are also 2 subscales measuring family functioning: Daily Activities (3 items) and Family Relationships (5 items).

Item Generation and Tool Development: The tool was reported to have been developed via focus groups, cognitive interviews using protocols from the development of the original measurement model as well as clinical experience with chronically ill children and their families. There was no description as to the number of participants in the item generation and tool development phase. The tool was field tested in a study that examined the healing environment at a convalescent hospital.

Scoring: The scales are consistent with those in the PedsQL4.0™ generic core scales, with 5 Likert response options: Never, Almost Never, Sometimes, Often, and Almost Always. These are reverse scored and linearly transformed as 100, 75, 50, 25, and 0 respectively. Higher scores indicated better functioning as a family and a reduced negative impact. If greater than 50% of the items were missing then it was suggested not to total scale scores. There are 3 summary scores: Total Score, Parent QOL Summary Score and the Family Functioning Summary Score. The Total Score of family impact is calculated as the mean of every item answered. The Parent QOL Summary score is the mean of the 20 items from the Physical, Emotional, Social and Cognitive Functioning Scales. The Family Functioning Summary Score is the mean of 8 items from the Daily Activities and Family Relationships scales.
Feasibility: Feasibility was not assessed in the preliminary article on reliability and validity of this tool. A study by Panepinto et al. in 2009 examined the psychometric properties in children with and without Sickle Cell Disease. A convenience sample of parents of children was recruited as part of a larger study of health-related QOL. Ninety-seven proxies of children with Sickle Cell disease and 73 proxies from the control group completed the survey. Moderate to high ceiling effects were reported for the Emotional, Social, and Cognitive Functioning, Communication, Daily Activities and Family Relationship scales. In addition, for the control group there was a moderate ceiling effect in the Family Functioning Scale and the Worry Scale. There was no data reported on missing responses or range of responses.

Validity: Construct validity was assessed using the known-groups method in the preliminary psychometric analysis by Varni et al., a Sickle Cell Disease study by Panepinto et al. and a Brazilian study that translated the tool into Portuguese and tested it in families of children with cancer. Varni et al. hypothesized that the Family Impact module scores would be lower in a group of chronically ill children who were being cared for as outpatients (n=12) in comparison to those who were cared for at an inpatient convalescence hospital (n=11). Despite the small sample size, there were significant differences between 7 of 11 scores, excluding Emotional Functioning, Worry, Family Relationships and the Family Functioning Summary score. These results were similar to those for the Brazilian study where they hypothesized that 29 families of children treated as inpatients would have higher scores (less negative impact) then the 66 families of children receiving treatment as an outpatient. They found medium to large effect sizes for all scales except Cognitive Functioning and Daily Activities. Both studies concluded that generally the results provided evidence of construct validity. Panepinto et al. hypothesized that the 97 proxy assessments of the children with Sickle Cell Disease would be lower than the 73 proxy respondents in the control group whose children did not have Sickle Cell disease. There were significantly lower scores in the 3 scales including Communication, Worry, and Daily Activities scales with mild or moderate effect sizes. They also repeated the comparison excluding children in the control group who had any co-morbidities in order to ensure a healthy cohort. Statistical differences were found only in Worry and Communication scales, however it was noted that the proxy assessments of those with Sickle Cell Disease and controls categorized as having medical and neurobehavioral co-
morbidities tended to have lower scores\textsuperscript{102}. There was less evidence in this population supporting construct validity.

Panepinto et al. also conducted an exploratory factor analysis and found that the factor structure deviated from the theoretical expectations in the Emotional and Social Functioning, Daily Activities and Communication scales\textsuperscript{102}.

**Reliability: Internal consistency reliability** was assessed in the studies by Varni et al., Panepinto et al. and Scarpelli et al.\textsuperscript{102,151,152} Guidelines to declare evidence of reliability were according to Nunnally's suggestion\textsuperscript{115}. In both studies, all scales had Cronbach's alpha greater than 0.80\textsuperscript{102,151}. There were a number of scales, including the summary scores that were well above 0.90 indicating that they were appropriate for individual analysis however potentially indicating item redundancy\textsuperscript{46}. The results of internal consistency in the Brazilian study did not result in Cronbach's alpha that were greater than 0.90 indicating it was not appropriate for individual testing but most scales analyzed for the total sample were greater than 0.70\textsuperscript{152}. However, there were 4 scales less than 0.70 demonstrating poor internal consistency\textsuperscript{152}. Test-retest reliability was conducted with 47 subjects in the Brazilian study with 7 days as the duration between testing\textsuperscript{152}. Correlation coefficients were high (0.81 to 0.96) for all subscales.

**Normative Data Comparison:** At the time of this study, there were no published normative values available for comparison.

This is a relatively new tool that did not have published normative data available for comparison given that the tool is intended to assess families with a chronically ill child. It is one of the only tools available to assess both the impact on parent and family function in children with chronic illness. There is some literature supporting construct validity, internal reliability and test-retest reliability however more data to validate its psychometric properties is needed. In addition, the methods for its development should have been outlined more clearly.

**4.2.1.6 Published Small Bowel Transplant Data**

The results from the generic QOL tools were compared to two sets of published data for SBTx patients. The first SBTx sample was studied in Nebraska, a study published in the American Journal of Transplantation in 2004 by Sudan et al\textsuperscript{18}. The Nebraska sample of intestinal
transplant recipients had a mean age of 10.6 ± 3.6 years with 9 male and 13 female respondents, and 16 of 22 children were in elementary school (Grade 1 to 8). Twelve patients received an isolated small bowel graft and 10 received a combined liver and small bowel graft. The mean length of time since transplant in years was 4.5 ± 2.2. Sudan et al. administered the proxy report CHQ-PF50 to 22 participants and the self report CHQ-CF87 to 21 participants and presented the results as means ± SD.

The second sample for comparison was in an abstract presented by Ngo et al. at the 2009 International Small Bowel Transplant Symposium. The results presented were those of a proxy assessment of 33 intestinal transplant patients followed at a Children’s Hospital in Los Angeles, California (13 males and 20 females). The mean age at transplant was 7.5 ± 5.8 years. These children had been transplanted a mean of 3.2 ± 2.1 years ago. A mean age for the sample was not provided. Ngo et al. solely investigated parent-proxy using the CHQ-PF50 and the PedsQL4.0 Proxy Report.

4.2.2 QUALITATIVE OUTCOME MEASURES

4.2.2.1 Focus Groups

Many individuals involved in qualitative research argue that classic measurement models cannot be used to assess qualitative research – i.e. reliability and validity, because there are no correct answers to use as a comparison. Winkler suggested asking respondents themselves to validate the determined themes since they essentially established these themes. Guba and Lincoln proposed 4 criteria to substitute for the traditional quantitative criteria (See Table 4.1). Table 4.1 Qualitative Criteria by Guba and Lincoln

<table>
<thead>
<tr>
<th>Quantitative Criteria</th>
<th>Qualitative Substitute</th>
<th>Definition</th>
</tr>
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<tbody>
<tr>
<td>Internal Validity</td>
<td>Credibility</td>
<td>Are the results believable from the perspective of the participant since it is from their eyes?</td>
</tr>
<tr>
<td>External Validity</td>
<td>Transferability</td>
<td>Can you transfer or apply these results to other settings or contexts? It is very important to describe context and assumptions.</td>
</tr>
<tr>
<td>Reliability</td>
<td>Dependability</td>
<td>Describe the changes in setting and how these may have affected research.</td>
</tr>
<tr>
<td>Objectivity</td>
<td>Confirmability</td>
<td>Confirmation of the results by checking and rechecking data (may consider having someone play devil’s advocate).</td>
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</tbody>
</table>
Other qualitative researchers maintained that utilizing this type of criteria suggests that there is a reality to the research which is an opposing view of true qualitative research. In this study, the above criteria will be informally reviewed in the discussion. Confirmability will be assessed by agreement of the devised themes by the 3 facilitators of the focus groups.

The focus groups allowed parent proxies to come together to share experiences, opinions and support for feelings. Parents were informed that the role of the focus group was to elicit their perceptions and feelings about the home PN experience. The participants were encouraged to discuss their experiences related to home PN with each other rather than with the researchers and to exchange stories and examples in order to enhance meaning. In the focus group, the aim was to remain the least directive by using encouraging statements and body language, reflecting on remarks made by the informant and only if necessary, probing on the last remark by the participant. Several open-ended questions had been prepared for all sessions in order to help the primary facilitator guide the focus group (See Appendix F - Focus Group Preparation).

Kitzinger described this as a moderately structured group funnel approach where in the middle of the funnel, three or four broadly defined central topics are introduced and the participants speak to these issues. Information obtained from the quantitative surveys completed in phase 1 of the study was discussed in the focus groups to promote further understanding of important issues. The focus groups provided the researchers, through group interaction, an opportunity to find out what these participants and their families experience having a child with intestinal failure on home PN.

4.3 Data Collection Forms

A co-investigator was responsible for the management of data collection. The data was collected and documented on paper case report forms (CRF) that were created for the study (See Appendix D - Case Report Form). Each subject had a unique study database ID not allowing for personal identification. A master list of study ID’s was maintained by the primary investigator which was double password-protected on a password-protected computer in a secure office. Data was entered from the CRF’s and the completed questionnaires into a secure password-protected Microsoft Excel file designed for the study, which was on a computer in a locked office. Transcription of the focus group data was completed by a professional
transcriptionist and the transcribed data was kept in a password-protected Microsoft Word file on a password-protected computer in a secure office.

4.4 Statistical Analysis

Statistics were analyzed using SAS Version 9.1.3 (SAS Institute, Cary, NC). All data was assessed for normality using the *proc univariate* command in SAS, specifically by review of skewness measurements and the Kolmogorov-Smirnov (KS) test. The KS test represents the largest absolute difference between the observed distribution and theoretical cumulative distribution function. Significantly skewed or non-normal data was analyzed using non-parametric statistics, unless comparative data was presented as parametric data. The level of significance was set at a p value of less than 0.05.

4.4.1 SUMMARY STATISTICS

Descriptive statistics were used to describe participant medical and demographic data using means with standard deviations and ranges and/or medians with interquartile ranges.

4.4.2 PRIMARY ANALYSIS

4.4.2.1 Primary Quantitative Analysis

The primary analysis was a comparative analysis of the generic QOL tools versus published norms and published SBTx data. The ITQOL and PedsQL™ Self Report were compared to published norms only as there was no SBTx data for comparison. The CHQ-PF50 proxy report, CHQ-CF87 child self report and PedsQL™ Proxy Report were compared to both normative and SBTx data. The results for the PedsQL4.0™ age-specific tools were analyzed cumulatively in order to compare to the data in the normative study, which was also presented in this manner.

The comparative analysis of the scale and summary scores was accomplished using independent 2 sample t-tests expressed as mean values +/- standard deviation. 95% confidence intervals (CI) around the mean differences were calculated using mean scores and variances. Significance was determined according to a p-value of <0.05. Effect sizes were calculated as follows using an
online effect size calculator\textsuperscript{157}:

\[
\text{Effect Size (Cohen’s } d \text{)} = \frac{\text{Mean of Comparison Group – Mean of Sample}}{\text{Pooled Standard Deviation}}
\]

Effect sizes are descriptive measures used to assess the magnitude of effect\textsuperscript{113} and are independent of sample size unlike tests of significance. To assess the clinical importance between the mean differences, effect sizes were assessed according to Cohen’s guidelines:

- Small = 0.2 ≤ \( \hat{d} \) < 0.5;
- Moderate = 0.5 \( \leq \hat{d} < 0.8; \) and Large = \( \hat{d} \geq 0.8 \textsuperscript{129}.

Effect sizes may be positive or negative, as that simply relates to the directionality of effect. Controversy exists in terms of what a significant magnitude of difference in scale scores is when measuring QOL and many researchers are of the opinion that what is found to be non-significant is not equivalent to insignificant on a clinical basis\textsuperscript{113}.

In this study, there were small sample sizes which may have resulted in fewer significant findings. Thus, for the home PN sample, a descriptive analysis of mean differences and a qualitative approach was used to assist with the interpretation of quantitative findings.

\subsection*{4.4.2.2 Primary Qualitative Analysis}

In view of utilizing a non-supported focus group, this study focused on an exploratory analysis in order to generate hypotheses. Qualitative data was analyzed using thematic analysis, which is thought to be a foundational method for qualitative analysis\textsuperscript{158}. Some researchers state that it is only a part of the major analytic traditions such as grounded theory however Braun and Clarke argue that it is its own method\textsuperscript{158}. In fact, it is quite common for papers to claim other theories (e.g. phenomenological theory) when they are actually using thematic analysis. Thematic analysis was defined by Braun as a method for identifying, analyzing and reporting patterns (themes) within data\textsuperscript{158}. It is an active process of identifying patterns and themes and selecting those that are reported requiring less pre-defined organization while still describing the data in a rich manner. The disadvantages of thematic analysis, according to Braun, are related to poor analysis, inappropriate research questions and flexibility if the intention is to do a more focused analysis. Thematic analysis can be utilized for the following theoretical positions:
realist/essentialist method, constructionist method, and contextualist method. The thematic analysis was a realist approach, reporting the realities, experiences and meanings of the participants in the focus group.

The focus groups were audio-taped and transcribed verbatim by a professional transcriptionist. Pauses, silences, laughter, and/or crying was documented in the field notes and coded in parentheses on the transcript to help to identify knowledge and feelings. The names of the participants and their children were eliminated from the transcript and pseudonyms were developed in order to maintain confidentiality. A co-investigator analyzed the transcribed data from the focus groups and the data-derived codes were systematically applied by assessing the occurrence of common themes. Data items were extracted for feature in the final analysis. The analysis included identification of common themes however; the prevalence for each theme was flexible and related to the research question. In order to improve the validity, the overall themes were reviewed and adjusted in conjunction with an experienced qualitative researcher and the primary facilitator of the focus group.

4.4.3 SECONDARY ANALYSIS

The secondary quantitative analysis encompassed the following:

i. A comparative analysis of ITQOL, CHQ (PF50, CF87) and PedsQL4.0™ scores in home PN patients versus institutional SBTx patients using non-parametric testing of two samples (Wilcoxon-Rank Test) and expressed as median values and interquartile ranges.

ii. A comparative analysis of Functional Status II(R) scores in home PN patients versus published norms and institutional SBTx populations using 2 sample t-tests expressed as mean values +/- standard deviation. 95% confidence intervals of the mean differences were calculated and significance corresponded to a p-value of <0.05.

iii. Assessment of VAS results (overall QOL, general health and pain) in home PN patients versus institutional SBTx populations using Wilcoxon Rank testing between the two-samples. A study done by Maxwell in 1978 looked at whether VAS data should be considered as parametric or non-parametric data and found little difference in which statistical technique was used however, Nyren (1988) stated that VAS measurements violated parametric assumptions and thus non-parametric testing was used for...
analysis. VAS scores were also assessed categorically as high (≥8cm) or low (<8cm) scores\textsuperscript{128,161} and analyzed using Fisher\textsuperscript{a} exact tests to assess statistically significant differences at a level of \( p < 0.05 \). VAS results were presented in terms of medians with interquartile ranges, mean differences as continuous data as well as percentages for the categorical classification.

iv. Analysis of each quantitative questionnaire administered to examine response rates, completion of tool, ceiling & basement effects.

### 4.5 Sample Size

This was a cross-sectional study which, for reasons of feasibility, had a fixed sample size of approximately 16 subjects in the home PN program and 6 patients in the SBTx program.

A two-tailed two sample test power calculation was done using the main QOL questionnaire ï the CHQ - and it was determined that a 10-point change in score was considered to be significant, which has been suggested as a rule of thumb to apply on 100-point QOL scales\textsuperscript{162}. The calculation was repeated using a point difference of 20 in the article by Sudan et al\textsuperscript{18}. See Table 4.2 for Power Calculations.

**Table 4.2 Power Calculations**

<table>
<thead>
<tr>
<th></th>
<th>Group 1 - Intestinal failure on home PN</th>
<th>Group 2 - Published SBTx\textsuperscript{18}</th>
<th>Home PN vs. CHQ PUBLISHED NORMS</th>
</tr>
</thead>
<tbody>
<tr>
<td>Home PN vs. SMALL BOWEL TRANSPLANT</td>
<td>n = 16</td>
<td>n = 22</td>
<td>ESTIMATED STANDARD DEVIATION (SD)</td>
</tr>
<tr>
<td><strong>Using Mean Point Difference = 10</strong></td>
<td></td>
<td></td>
<td>SD = 10</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Power = 84.2%</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Power = 50.6%</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Power = 31.6%</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>SD = 15</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Power = 97.5%</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Power = 74.2%</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Power = 49.9%</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>SD = 20</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Power = 100%</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Power = 97.7%</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Power = 84.2%</td>
</tr>
<tr>
<td>Home PN vs. SMALL BOWEL TRANSPLANT</td>
<td>n = 16</td>
<td>n = 22</td>
<td>ESTIMATED STANDARD DEVIATION (SD)</td>
</tr>
<tr>
<td><strong>Using Mean Point Difference = 20</strong></td>
<td></td>
<td></td>
<td>SD = 10</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Power = 100%</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Power = 100%</td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td>Power = 97.5%</td>
</tr>
</tbody>
</table>
Participants were recruited at the Hospital for Sick Children in the GIFT, Clinical Nutrition and SBTx outpatient clinics. Patient recruitment occurred between January and May 2010. See Figure 5.1 for Study Recruitment Diagram.

The patients on home PN who met eligibility criteria were approached for study participation in clinic (n=15). Home PN patients who were living out of province were contacted via telephone in order to introduce the study and to provide the opportunity to participate (n=3). However, contact was made with only 1 of 3 patients living out of province. Instructions, surveys and
consent forms were emailed to this family and were mailed back to the co-investigator. One particular adolescent agreed to participate in the study in clinic, however the parent would not consent at the request of the child. With respect to survival, there have been no deaths in the home PN group since 2006. The post SBTx patients were recruited from the SBTx outpatient clinic. There were 7 surviving children post SBTx of 14 SBTx performed at this institution. All surviving patients who had received a SBTx (isolated, small bowel-liver combined and/or multivisceral transplants) were approached for study participation (n=7). One family who was living out of province was contacted by telephone. Instructions, surveys and consent were mailed to the family and successfully returned.

In the Home PN group, 16 of 16 packages were returned with a response rate of 100%. In the SBTx group, 6 of 7 families returned the surveys with a response rate of 85.7%.

Seven families were asked to participate in the focus groups as proxies for the home PN patients and 6 parents agreed to participate, representing 5 children on home PN. Due to the lack of age-appropriate children on home PN who were available and lived locally, there was not an opportunity to conduct a focus group with the patients themselves.

5.2 Participant Demographic and Clinical Data

Demographic and clinical characteristics of patients were gathered to describe the home PN and SBTx patient populations. The demographic data is summarized in Table 5.1. Normality was assessed and all continuous variables were normal excluding: home PN duration, home PN age at initiation, percent of calories from enteral and parenteral nutrition and number of days in hospital over a six month and one year period. Means with standard deviations (SD) and ranges as well as medians with interquartile ranges (IQR) are presented where appropriate.
Table 5.1 Demographic Characteristics of Home PN and SBTx Participants

<table>
<thead>
<tr>
<th>Demographic Parameter</th>
<th>Home PN (n=16)</th>
<th>SBTx (n=6)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Gender</td>
<td>Male/Female (%Male/%Female)</td>
<td>11/5 (68.8/31.2)</td>
</tr>
<tr>
<td>Age (years)</td>
<td>Mean (SD)</td>
<td>6.1 (6.0)</td>
</tr>
<tr>
<td></td>
<td>Range</td>
<td>0.6 – 18.2</td>
</tr>
<tr>
<td>Patient Education</td>
<td>Infant/Toddler (Home)</td>
<td>8 (50.0%)</td>
</tr>
<tr>
<td></td>
<td>Daycare</td>
<td>1 (6.2%)</td>
</tr>
<tr>
<td></td>
<td>Kindergarten</td>
<td>1 (6.2%)</td>
</tr>
<tr>
<td></td>
<td>Grade 1-8</td>
<td>4 (25.0%)</td>
</tr>
<tr>
<td></td>
<td>Grade 9-12</td>
<td>2 (12.6%)</td>
</tr>
<tr>
<td></td>
<td>University/College</td>
<td>0</td>
</tr>
<tr>
<td>Parent Marital Status</td>
<td>Married/Living Together</td>
<td>15 (93.8%)</td>
</tr>
<tr>
<td></td>
<td>Divorced</td>
<td>0</td>
</tr>
<tr>
<td></td>
<td>Single/Never married</td>
<td>1 (6.2%)</td>
</tr>
<tr>
<td>Income</td>
<td>Single Parent</td>
<td>8 (50.0%)</td>
</tr>
<tr>
<td></td>
<td>Dual Parent</td>
<td>7 (43.8%)</td>
</tr>
<tr>
<td></td>
<td>None working</td>
<td>1 (6.2%)</td>
</tr>
<tr>
<td>Drug Benefits</td>
<td>Receive Benefits</td>
<td>16 (100%)</td>
</tr>
<tr>
<td>Use of Social Assistance</td>
<td>Yes</td>
<td>2 (12.5%)</td>
</tr>
<tr>
<td>Distance to Travel to SickKids</td>
<td>Local GTA</td>
<td>8 (50.0%)</td>
</tr>
<tr>
<td></td>
<td>&lt;100km</td>
<td>3 (18.8%)</td>
</tr>
<tr>
<td></td>
<td>&gt;100km</td>
<td>4 (25.0%)</td>
</tr>
<tr>
<td></td>
<td>Out of Province</td>
<td>1 (6.2%)</td>
</tr>
</tbody>
</table>

The mean age of the Home PN group was 6.1 with a range of 0.6 to 18.2 years with 11 boys (68.8%) and 5 girls (31.2%). The SBTx group consisted of 4 boys (66.7%) and 2 girls (33.3%) with a mean age of 5.5 years and a more narrow age range, 3.4 to 9.8 years. Fifty percent of the home PN population were infants/toddlers and had not yet started daycare or school, in contrast to 50% of the SBTx population who were in kindergarten or grade 1. Both groups of patients primarily came from intact families, received drug benefits and were not currently in use of social assistance. Half of the home PN sample and 4 of 6 families in the SBTx program had a single parent income related to mothers on maternity leave for infants on home PN or those who chose to stay home to take care of their children. Fifty percent or more of families from both groups lived within the local Greater Toronto Area (GTA) or within 100km of the hospital. The clinical characteristics of the recruited population are found in Table 5.2.
<table>
<thead>
<tr>
<th>Clinical Parameter</th>
<th>Home PN (n=16)</th>
<th>SBTx (n=6)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Primary Diagnoses</strong></td>
<td>Tufting Enteropathy (1)</td>
<td>Microvillus Inclusion Disease (1)</td>
</tr>
<tr>
<td></td>
<td>Lymphangiectasia (1)</td>
<td>SBS secondary to:</td>
</tr>
<tr>
<td></td>
<td>Other (3)</td>
<td>• Abdominal Wall Defects (1)</td>
</tr>
<tr>
<td></td>
<td>Abdominal Wall Defects (4)</td>
<td>• Intestinal Atresia (2)</td>
</tr>
<tr>
<td></td>
<td>• Intestinal Atresia (1)</td>
<td>• Volvulus (1)</td>
</tr>
<tr>
<td></td>
<td>• Hirschsprung's Disease (5)</td>
<td>• Necrotizing Enterocolitis (1)</td>
</tr>
<tr>
<td><strong>Presence of ostomy</strong></td>
<td>Yes/No (%)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>8/8</td>
<td>1/5</td>
</tr>
<tr>
<td></td>
<td>50/50</td>
<td>16.7/83.3</td>
</tr>
<tr>
<td><strong>Presence of Drains</strong></td>
<td>Yes/No (%)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>2/14</td>
<td>0/6</td>
</tr>
<tr>
<td></td>
<td>12.5/87.5</td>
<td>0/100</td>
</tr>
<tr>
<td><strong>Enteral Feeding Tube In Situ</strong></td>
<td>Nasogastric</td>
<td></td>
</tr>
<tr>
<td></td>
<td>Gastrostomy</td>
<td></td>
</tr>
<tr>
<td></td>
<td>Nasojejunal</td>
<td></td>
</tr>
<tr>
<td></td>
<td>Gastrojejunal</td>
<td></td>
</tr>
<tr>
<td></td>
<td>Jejunostomy</td>
<td></td>
</tr>
<tr>
<td></td>
<td>None</td>
<td></td>
</tr>
<tr>
<td><strong>Number of Daily Medications</strong></td>
<td>Mean (SD)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>Range</td>
<td></td>
</tr>
<tr>
<td></td>
<td>3.5 (2.2)</td>
<td>5.7 (2.3)</td>
</tr>
<tr>
<td></td>
<td>0-7</td>
<td>2-9</td>
</tr>
<tr>
<td><strong>Number of Daily Medication Doses</strong></td>
<td>Mean (SD)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>Range</td>
<td></td>
</tr>
<tr>
<td></td>
<td>6.6 (4.8)</td>
<td>9 (3.0)</td>
</tr>
<tr>
<td></td>
<td>0-17</td>
<td>4 - 13</td>
</tr>
<tr>
<td><strong>Weight (Z-scores)</strong></td>
<td>Mean (SD)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>Range</td>
<td></td>
</tr>
<tr>
<td></td>
<td>-1.048 (1.35)</td>
<td>-0.305 (1.93) *</td>
</tr>
<tr>
<td></td>
<td>-4.286 ÷ 0.688</td>
<td>-3.603 ÷ 1.072 *</td>
</tr>
<tr>
<td><strong>Height (Z-scores)</strong></td>
<td>Mean (SD)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>Range</td>
<td></td>
</tr>
<tr>
<td></td>
<td>-1.11 (1.38)</td>
<td>-1.004 (1.90) *</td>
</tr>
<tr>
<td></td>
<td>-3.946 ÷ 0.938</td>
<td>-4.303 ÷ 0.217 *</td>
</tr>
<tr>
<td><strong>Feeding Orally</strong></td>
<td>Yes/No (%)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>15/1</td>
<td>6/0</td>
</tr>
<tr>
<td></td>
<td>93.8/6.2</td>
<td>100/0</td>
</tr>
<tr>
<td><strong>Presence of Oral Aversion (Y/N)</strong></td>
<td>Yes/No (%)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>2/14</td>
<td>0/6</td>
</tr>
<tr>
<td></td>
<td>12.5/87.5</td>
<td>0/100</td>
</tr>
<tr>
<td><strong>Enteral Tube Feeds</strong></td>
<td>Receiving feeds (%)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>6/10</td>
<td>3/3</td>
</tr>
<tr>
<td></td>
<td>37.5/62.5</td>
<td>50/50</td>
</tr>
<tr>
<td><strong># Hrs on Feeds</strong></td>
<td>Mean (SD)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>Range</td>
<td></td>
</tr>
<tr>
<td></td>
<td>9.2 (2.6)</td>
<td>15.3 (12.0)</td>
</tr>
<tr>
<td></td>
<td>5-13</td>
<td>10-24</td>
</tr>
<tr>
<td><strong>% kcals (including oral)</strong></td>
<td>Mean (SD)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>Range</td>
<td></td>
</tr>
<tr>
<td></td>
<td>57.6 (32.4)</td>
<td>90.0 (24.5)</td>
</tr>
<tr>
<td></td>
<td>0 ÷ 90</td>
<td>40-100</td>
</tr>
</tbody>
</table>

*Based on n=5
†Based on n=1

Table 5.2 continued on next page.
Table 5.2 Clinical Characteristics of Home PN and SBTx Participants

<table>
<thead>
<tr>
<th>Clinical Parameter</th>
<th>Home PN (n=16)</th>
<th>SBTx (n=6)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Parenteral Nutrition</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td># Hours on PN/day</td>
<td>Mean (SD) 12.0 (2.9)</td>
<td>12  ý</td>
</tr>
<tr>
<td>Range</td>
<td>8-18</td>
<td></td>
</tr>
<tr>
<td>%PN Kcals</td>
<td>Mean (SD) 42.4 (32.4)</td>
<td>60  ý</td>
</tr>
<tr>
<td>Range</td>
<td>10-100</td>
<td></td>
</tr>
<tr>
<td># days on PN</td>
<td>3 days 1, 4 days 1, 6 days 1, 7 days 13 (81.3%)</td>
<td>7  ý</td>
</tr>
</tbody>
</table>

| **Advanced Liver Disease (C Bili >100umol/L)** | Present | 0 |
| Listened for Transplant | Yes | 0 (87.5%) |
| No | 14 (87.5%) |
| On Hold | 2 (12.5% - combined SB/liver) |

| **Duration on Home PN (years)** | Median 0.94 | 1.00  ý |
| IQR | 0.33 – 3.43 | |
| Range | 0.17 – 17.04 | |

| **Age at Home PN initiation (years)** | Median 0.91 | 8.80  ý |
| IQR | 0.42 – 1.88 | |
| Range | 0.23 – 17.46 | |

| **Past Home PN** | Yes/No | 2/14 | 3/3 |
| **Age at First Transplant (years)** | Mean (SD) 0.7 ý | 1.4 (0.96) |
| Range | | 0.6 – 3.0 |

| **Time Since First Transplant** | <1 year | 2 (33.3%) |
| 1-3 years | 0 (16.7%) |
| >3 years | 4 (66.7%) |

| **Type of First Transplant** | Isolated Small Bowel Tx | 1 |
| Combined Liver/Bowel Tx | 0 |
| Isolated Liver Tx | 5 (83.3%) |

| **Retransplantation** | Yes | 0 |

| **Meds for Anxiety/Depression** | Yes/No (%) | 2/14 (12.5/87.5) | 0/6 |
| **Meds for Pain** | Yes/No (%) | 1/15 (6.3/93.7) | 0/6 |

| **# of days in hospital in past 6 months** | Median 4.5 | 2 |
| IQR | 1-33 | 0-5 |
| Range | 0-141 | 0-14 |

| **# of days in hospital in past 1 year** | Median 32.5 | 15 |
| IQR | 8.5-92 | 2-15 |
| Range | 0-258 | 0-34 |

*based on n=5
ýbased on n=1
In this study, the primary underlying diseases resulting in intestinal failure in both populations was short bowel syndrome (SBS) secondary to multiple etiologies including abdominal wall defects, long segment Hirschsprung’s disease, necrotizing enterocolitis, volvulus and intestinal atresias. In the home PN population, the most common underlying cause of intestinal failure was SBS related to long segment Hirschsprung’s disease (n=5; 31%) and abdominal wall defects comprised another 25%. Half of the children on home PN had an ostomy compared to only one child in the SBTx sample. The children with an enteral feeding tube in situ mainly had gastrostomy tubes for both enteral feed and medication administration.

Weight and height z-scores are linear, sex-independent and allow calculation of summary statistics. The z-scores indicate how far the child deviates from the average. The mean z-scores for height and weight were approximately 1 SD below zero which is within normal. A z-score of -2 for weight indicates underweight and for height indicates stunting. There were children in both groups who were stunted in height and underweight compared to references.

One child who participated in the study from the SBTx group had PN restarted post-transplant following an episode of severe rejection. At the time of the study, this child was receiving PN 7 days a week, infusing over 12 hours per day. The home PN sample was administered PN 3 to 7 days per week with 81.3% of the children receiving PN daily. PN was infused over 8 to 18 hours per day and provided 10-100% of total caloric requirements in this sample. All children in both groups received oral feeds and two home PN children had a documented oral feeding aversion.

The median age and overall age range related to initiation and duration of home PN was approximately 1 year (range: 0.2-17 years). There were no participants with a conjugated bilirubin of 100 umol/L or greater that would be classified as advanced liver disease or severe IFALD. In comparison, 5 of the 6 SBTx subjects received a liver in conjunction with the transplanted intestine and one child in the home PN group required a liver transplant 3 years ago all for the indication of severe IFALD.

The children who received a SBTx had a mean age at transplant of 1.4 years, ranging from 0.6 to 3 years of age. Four of the children were greater than 3 years from the date of their SBTx and 2
children received their SBTx within the last 1 to 3 years. None of the children in this sample required a second transplant. One child in the home PN group received an isolated liver transplant for the indication of severe IFALD, greater than 3 years ago.

The median number of days that the home PN children spent as an inpatient in the last six months and one year, were 4.5 and 32.5 days, consecutively (range: 0-141 days in 6 months and 0-258 days in 1 year) whereas the SBTx children spent a median of 2 and 15 days, consecutively (range: 0-14 days in 6 months; 0-34 days in 1 year). In the home PN sample, 2 children were taking medication for anxiety or depression and 1 child was on pain medication. Neither type of medication was being used in the SBTx sample.

Due to the minimal number of non-responders, the responders and non-responders were not statistically compared. Eighty-nine percent of the eligible home PN population and 86% of the SBTx population participated in the study demonstrating representativeness in both groups.

5.3 Primary Quantitative Analysis

Scale scores from the generic proxy QOL questionnaires (ITQOL, CHQ-PF50, PedsQL™ Proxy Report) and the self-reports (CHQ-CF87, PedsQL™ Self Report) were calculated for respondents who had completed ≥50% of the items. According to this guideline, all scales on the returned questionnaires were complete. The scale scores were all scored according to the publisher’s guidelines and resulted in a score from 0 (worst) to 100 (best), higher scores being more favourable. The results of these surveys were compared to published normative values and published SBTx data where available. A table summarizing the overall results of the battery assessment of proxy respondents and the comparisons can be found in Appendix J and the self-report summary can be found in Appendix K.
5.3.1 COMPARISON OF GENERIC QOL SURVEY RESULTS OF HOME PN SAMPLE TO NORMATIVE DATA

5.3.1.1 Proxy Reports

5.3.1.1.1 Infant Toddler Quality of Life (ITQOL) Questionnaire

The ITQOL is a proxy-based assessment of QOL in children aged 2 months to 5 years. In our sample of home PN patients, 10 parents completed the ITQOL. Only 4 questionnaires were available for analysis of the Physical Functioning scale and 7 were available for scales intended for children greater than 1 year of age. The Physical Functioning scale has an option of ‘not doing yet’ for parents to choose if their child is not old enough to be performing a specific activity developmentally. If that option is chosen, it is recommended by the author to exclude these from the analysis. The normative data was available for comparing all scales on the ITQOL but excluded the 2 global items for health and behaviour. Summarized results comparing the home PN population with the published norms can be found in Table 5.3.

Using independent sample t-tests, there was a significant difference found between the home PN sample and published norms on the children’s Physical Functioning, Growth and Development, Bodily Pain and General Health Perceptions scales, with the home PN group scoring lower on these domains. Effect sizes were calculated and found to be of large effect for each of these scales. The largest absolute mean difference and the largest effect size related to the General Health Perceptions scale. There was a wide range of responses on this scale from 11.4 to 63.6 out of 100. However even the highest score in the home PN sample was less than the mean score for the published norms (79.0). This scale included 12 items that reflect the previous and current health status of the child and their perceived health in the future. One home PN respondent noted on the side of the survey that they thought their child might die in the first 7 months that they were in hospital but they worry less now because they ‘seem healthy’. The Physical Functioning scale was rated by 4 respondents as significantly lower (p value<0.0001) in the home PN group despite a wide range of scores (60-97 out of 100). This asked families to rate their child’s limitations related to feeding, playing, walking etc. Some comments from the home PN families included difficulties with feeding related to their child’s inability to drink ‘normal’ formula. One mother wrote that her child was limited in playing due to ‘Long hours of TPN IV infusion’. In terms of physical activity, a mother expressed that their child’s limitations relating
to taking steps or walking was related to developmental regression after an extended hospital stay. The **Bodily Pain** scale in home PN was significantly different than the published norms and was 14 mean points lower with a small standard deviation. This particular scale reflected upon the past 4 weeks and asked families to assess pain based not only on their underlying illness but also teething, gas etc. Comments added to the surveys included pain attributable to non-PN related issues such as teething and a foot cast. However, another comment related pain to dressing and cap changes of the central line. There was no significant difference related to **Behaviour**. A comparison could not be calculated for the change in health scale that was a single item question reflecting upon the change in the child’s health over the past year. The result was calculated as a categorical variable and was not intended to be analyzed continuously. The normative value in the article by Raat et al. presented this variable as a number but did not detail how they modified the variable to be continuous. Of the 7 respondents, half indicated that they felt their child’s health was either somewhat better or much better than one year ago, and 2 respondents felt that their child’s health was either the same or much worse than one year ago. In the normative sample, the mean was close to 50 which they indicated to be representative of a similar perceived health (same as one year ago).

The other scales demonstrating statistical significance \((p < 0.0001)\) and large effect sizes included the **Parental Impact on Time and Emotion** scales, which represent the amount of emotional worry and concern expended and personal time available for the parents as a result of their child’s health and growth and development. The parents in the home PN population scored significantly lower than the published norms. See **Figure 5.2** for a graph of these results.

**Figure 5.2** Results of ITQOL Questionnaire \(\hat{1}\) Home PN versus Published Normative Data

\*indicates a statistically significant difference \((p \text{ value } <0.05)\) between the two samples
### Table 5.3 ITQOL Proxy Report Questionnaire - Home PN versus Published Normative Data

<table>
<thead>
<tr>
<th>ITQOL Scale</th>
<th>Home PN Sample Mean (SD) Range N=10</th>
<th>Published Norms Mean (SD) n=410</th>
<th>Mean Difference</th>
<th>Effect Size</th>
</tr>
</thead>
<tbody>
<tr>
<td>Global Health Item</td>
<td>50.0 (27.0) 0–85.0</td>
<td>n/a</td>
<td>-21.4*</td>
<td>-1.52</td>
</tr>
<tr>
<td>Physical Functioning</td>
<td>75.8 (17.3)b 60.0–96.7</td>
<td>97.2 (9.8)</td>
<td>-21.4*</td>
<td>-1.52</td>
</tr>
<tr>
<td>Growth and Development</td>
<td>73.5 (11.8) 50.0–92.5</td>
<td>86.5 (10.6)</td>
<td>-13.0*</td>
<td>-1.16</td>
</tr>
<tr>
<td>Bodily Pain</td>
<td>70.0 (10.5) 50.0–83.3</td>
<td>83.8 (16.8)</td>
<td>-13.8*</td>
<td>-0.99</td>
</tr>
<tr>
<td>Temperament and Moods</td>
<td>77.4 (8.6) 63.9–94.4</td>
<td>77.2 (10.5)</td>
<td>0.2</td>
<td></td>
</tr>
<tr>
<td>General Behaviour</td>
<td>73.5 (21.9)c 31.3–95.8</td>
<td>72.8 (12.7)</td>
<td>0.7</td>
<td></td>
</tr>
<tr>
<td>Global Behaviour Item</td>
<td>77.9 (25.0)c 30.0–100.0</td>
<td>n/a</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Getting Along</td>
<td>73.8 (8.8)b 63.3–88.3</td>
<td>71.4 (8.8)</td>
<td>2.4</td>
<td></td>
</tr>
<tr>
<td>General Health Perceptions</td>
<td>35.2 (19.3) 11.4–63.6</td>
<td>79.0 (14.5)</td>
<td>-43.8*</td>
<td>-2.57</td>
</tr>
<tr>
<td>Parental Impact: Emotion</td>
<td>71.8 (19.4) 39.3–100.0</td>
<td>92.1 (10.5)</td>
<td>-20.3*</td>
<td>-1.30</td>
</tr>
<tr>
<td>Parental Impact: Time</td>
<td>75.0 (18.3) 38.1–100.0</td>
<td>93.0 (11.0)</td>
<td>-18.0*</td>
<td>-1.19</td>
</tr>
<tr>
<td>Family Cohesion</td>
<td>77.0 (18.7) 60.0–100.0</td>
<td>75.3 (18.8)</td>
<td>1.7</td>
<td></td>
</tr>
<tr>
<td>Change in Health Ŷ</td>
<td>b Much worse ‑1 Somewhat worse ‑0 About same ‑1 Somewhat better ‑3 Much better ‑2</td>
<td>56.1 (18.4)</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

*a Physical Functioning Scale analysis utilized only complete scales (Home PN n=4)

*b Applicable for children >1 year only (Home PN n =7)

*c Reference for norms

† A score of 50 indicates a similar perceived health as 1 year ago, scores higher and lower than 50 indicate a better or worse perceived health, respectively

* statistically significant at p value <0.05

5.3.1.1.2 Child Health Questionnaire Parent Form 50 (CHQ-PF50)

The CHQ-PF50 was completed by proxy respondents for children greater than or equal to 5 years of age. The scoring is done in the same manner as the ITQOL according to the guidelines provided by the authors. The scale scores range from 0 to 100, higher being a more favourable score. A summary of results of the comparison between the 5 home PN proxy respondents and the 391 US school children norms can be found in Table 5.4. Consistent with the results from
the ITQOL, there was a statistically significant difference in scale scores and large effect sizes with the following domains: Physical Functioning, Role/Social Limitations Owing to Physical Function and Emotions/Behaviour, and General Health Perceptions. The home PN sample scored these domains significantly lower than the published norms. The **Physical Functioning** scale related to having difficulties with physical activities requiring energy due to the health of the child. The **Role and Social Limitations** related to the ability to participate in schoolwork and activities with friends as a result of physical, emotional and behavioural difficulties. This is in contrast with the negative result of Behaviour in the younger children rated on the ITQOL.

The **General Health Perceptions** scale as described above, relates to their perception and worry regarding their child’s past, present and current health status. A moderate effect size was present relating to statistically significant differences in the **Bodily Pain** scale (p = 0.042). This indicated a higher proxy perception of pain over the preceding 4 week period in the children on home PN compared to the normative population.

There was also a statistically significant difference in the Physical Functioning summary scale but not the psychosocial summary scale, consistent with non-significant findings in mental health, self esteem and behaviour scales. The **Self Esteem** scale mean was 12 points lower than the normative mean however; the standard deviation was quite large reflecting a wide range of scores (8.3 to 95 out of 100).

**Family Cohesion**, a single item asking how well the family gets along, was not statistically different compared to the normative population. The absolute mean difference between the two groups was 16.6 points with a non-significant higher mean score in the home PN sample compared to the normative population. The home PN group scored significantly lower (p<0.0001, large effect size -1.57) on the **Family Activities** scale which measured the perception that family activities were interrupted and/or limited over the last 4 weeks as a result of their child’s health. Large effect sizes and statistical significance were also present in terms of the **Parental Impact** scales in both **Time and Emotions**, similar to the above reported results from the ITQOL. Comparative information for the categorical variable change in health was unavailable however the home PN proxy perception of their child’s health compared to one year ago was that 2 were either somewhat better or much better, 2 were the same and 1 child was
worse than one year ago. See Figure 5.3 for a graphical representation comparing the Home PN group to the normative population.

**Table 5.4** CHQ-PF50 Proxy Report Home PN versus Published Normative Data

<table>
<thead>
<tr>
<th>CHQ-PF50 Scales</th>
<th>Home PN Sample Means (SD)</th>
<th>Published Norms Means (SD)</th>
<th>Mean Difference</th>
<th>Effect Size</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Range n=5</td>
<td>n=391</td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Physical Functioning</td>
<td>72.2 (31.7)</td>
<td>96.1 (13.9)</td>
<td>-23.9*</td>
<td>-0.98</td>
</tr>
<tr>
<td></td>
<td>16.7 ± 94.4</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Role/Social limitations Owing to</td>
<td>60.0 (38.4)</td>
<td>93.6 (18.6)</td>
<td>-33.6*</td>
<td>-1.12</td>
</tr>
<tr>
<td>Physical Function</td>
<td>0 ± 100.0</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>General Health</td>
<td>34.2 (9.9)</td>
<td>73 (17.3)</td>
<td>-38.8*</td>
<td>-2.75</td>
</tr>
<tr>
<td></td>
<td>26.7 ± 51.7</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Bodily Pain</td>
<td>64.0 (35.1)</td>
<td>81.7 (19)</td>
<td>-17.7*</td>
<td>-0.63</td>
</tr>
<tr>
<td></td>
<td>10.0 ± 100.0</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Role Limitations Owing to Emotions/Behavior</td>
<td>60.0 (43.5)</td>
<td>92.5 (18.6)</td>
<td>-32.5*</td>
<td>-0.97</td>
</tr>
<tr>
<td></td>
<td>0 ± 100.0</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Self esteem</td>
<td>68.2 (34.3)</td>
<td>79.8 (17.5)</td>
<td>-11.6</td>
<td></td>
</tr>
<tr>
<td></td>
<td>8.3 ± 95.0</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Mental Health</td>
<td>79.0 (19.2)</td>
<td>78.5 (13.2)</td>
<td>-1.4</td>
<td></td>
</tr>
<tr>
<td></td>
<td>45.0 ± 90.0</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Behavior</td>
<td>73.0 (17.0)</td>
<td>75.6 (16.7)</td>
<td>-2.6</td>
<td></td>
</tr>
<tr>
<td></td>
<td>51.7 ± 97.5</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Family Activities</td>
<td>54.2 (38.8)</td>
<td>89.7 (18.6)</td>
<td>-35.5*</td>
<td>-1.17</td>
</tr>
<tr>
<td></td>
<td>0 ± 95.8</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Family Cohesion</td>
<td>88.8 (7.5)</td>
<td>72.3 (21.6)</td>
<td>16.5</td>
<td></td>
</tr>
<tr>
<td></td>
<td>85.0 ± 100.0</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Parental impact on emotion</td>
<td>56.7 (35.1)</td>
<td>80.3 (19.1)</td>
<td>-23.6*</td>
<td>-0.84</td>
</tr>
<tr>
<td></td>
<td>8.3 ± 91.7</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Parental impact on time</td>
<td>60.0 (32.0)</td>
<td>87.8 (19.9)</td>
<td>-27.8*</td>
<td>-1.04</td>
</tr>
<tr>
<td></td>
<td>22.2 ± 100.0</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Global health</td>
<td>60.0 (0)</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Global behavior</td>
<td>78.0 (17.5)</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>60.0 ± 100.0</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Summary - Physical</td>
<td>32.2 (16.5)</td>
<td>53.0 (8.8)</td>
<td>-20.8*</td>
<td>-1.57</td>
</tr>
<tr>
<td></td>
<td>4.96 ± 49.2</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Summary - Psychosocial</td>
<td>45.5 (15.8)</td>
<td>51.2 (9.1)</td>
<td>-5.7</td>
<td></td>
</tr>
<tr>
<td></td>
<td>20.2 ± 59.8</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Change in Health</td>
<td>Much worse 0</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>Somewhat Worse 1</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>Same 2</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>Somewhat better 1</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>Much better 1</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

*a* Family cohesion scale incomplete (Home PN n=4)

*b* Reference for normative data

† Change in health † categorical data

*statistically significant at p value <0.05
Figure 5.3 Results of CHQ-PF50 Home PN versus Published Normative Data versus Published SBTx Data

*indicates statistical significance (p<0.05) comparing home PN to published norms

5.3.1.1.3 PedsQL4.0™ Proxy Report Questionnaires

The PedsQL4.0™ Proxy Report was completed by caregivers of children greater than or equal to 2 years of age. The results of the comparison between the proxy reports from the home PN sample (all ages) and the published norms can be found in Table 5.5. See Appendix L for results of home PN group separated by age group. Note that the Physical Summary score was omitted from the results as it is mathematically equivalent to the Physical Functioning scale score.

There were 9 respondents from the home PN group that were compared to the published norms by Varni et al.149 There was a statistically significant difference between the home PN sample and the published norms in means in all scales (Physical Functioning, Emotional Functioning, Social Functioning and School Functioning), the Psychosocial Summary score and the Total QOL Score. Large effect sizes were seen with each scale, except the Social Functioning scale which had a moderate effect size. The proxy reports of the home PN children scored significantly lower in all domains with most mean differences approaching 20 points or greater (out of a total of 100). The proxies scored these children based on an assessment over the past one month, similar to most time frames in the CHQ questionnaires. The significantly lower Emotional and Social Scales were in contrast to the results of the ITQOL and CHQ-PF50; however the Physical and School Functioning were consistent. In terms of Total QOL Scores,
the proxies scored the children on home PN 22 mean points lower than the normative population with similar standard deviations. See Figure 5.4 for results of the PedsQL4.0™ comparisons.

**Table 5.5** PedsQL4.0™ Proxy Report Questionnaire Home PN versus Published Normative Data

<table>
<thead>
<tr>
<th>PedsQL4.0™ Proxy Report Scales</th>
<th>Home PN Sample Mean (SD) Range n=9</th>
<th>Published Norms Mean (SD) n=9430</th>
<th>Mean Difference</th>
<th>Effect Size</th>
</tr>
</thead>
<tbody>
<tr>
<td>Physical Functioning Scale</td>
<td>59.4 (21.8) 21.9 – 87.5</td>
<td>84.5 (19.5)</td>
<td>-25.1*</td>
<td>-1.214</td>
</tr>
<tr>
<td>Emotional Functioning Scale</td>
<td>62.8 (16.6) 45.0 – 100.0</td>
<td>81.3 (16.5)</td>
<td>-18.5*</td>
<td>-1.118</td>
</tr>
<tr>
<td>Social Functioning</td>
<td>64.4 (28.4) 10.0 – 100.0</td>
<td>83.7 (19.4)</td>
<td>-19.3*</td>
<td>-0.794</td>
</tr>
<tr>
<td>School Functioning Scale</td>
<td>56.0 (28.4)b 20.0 – 90.0</td>
<td>78.8 (19.6)</td>
<td>-22.8*</td>
<td>-0.934</td>
</tr>
<tr>
<td>Psychosocial Summary Score</td>
<td>61.3 (19.2) 31.7 – 90.0</td>
<td>81.7 (15.2)</td>
<td>-20.4*</td>
<td>-1.178</td>
</tr>
<tr>
<td>Total Score</td>
<td>60.3 (18.3) 39.1 – 88.0</td>
<td>82.7 (15.4)</td>
<td>-22.4*</td>
<td>-1.324</td>
</tr>
</tbody>
</table>

*Reference for normative data

bBased on sample size n=8

**Figure 5.4** Results of PedsQL4.0™ Proxy Report Home PN versus Published Normative Data versus Published SBTx Data

*indicates statistical significance (p<0.05) comparing home PN to published norms.

Scales: PF-Physical Functioning, EF-Emotional Functioning, SF-Social Functioning, SC-School Functioning, PSS-Psychosocial Summary Score, TS-Total Score
5.3.1.2 Child Self-Report

5.3.1.2.1 Child Health Questionnaire Child Form 87 (CHQ-CF87)

The CHQ-CF87 was completed by children 10 years of age and older and was scored as per the author’s recommended guidelines. Scale scores range from 0 to 100, worst to best score. Independent 2 sample t tests were used to assess the mean difference between the children’s self rating scores in the home PN population (n=4) and the published normative data. The results of the comparison between the home PN group and the US school children norms can be found in Table 5.6. General Health Perceptions was the only scale with a statistically significant mean difference (p<0.0001) and a large effect size (-2.44). On this scale, the home PN group scored themselves 33 points lower than the published norms. This scale consisted of 12 items that reflected upon their perception of their past, present and future health concerns. This was consistent with ratings from the proxy assessment. Self Esteem was rated 11 points lower, albeit non-significantly, however the scoring ranged from 54 to 91. The same variability in responses occurred in Mental Health and Behaviour. All 4 children on home PN group scored the highest score (100/100) on the Role/Social Limitations Owing to Behaviour scale indicating their behaviour was not related to limitations in schoolwork and activities with friends. In contrast to the proxy results, there was no significant difference in the children’s assessment of their Physical Functioning compared to norms scoring within a narrow range (89.9-100 out of 100) and small SD (5.6). There was no normative data available for comparison of home PN in regards to Family Activities, Family Cohesion, global items (behaviour and health) or the Change in Health item. Two children on home PN believed their health was much better now and 2 others felt their health was the same or somewhat better than 1 year ago. None of these 4 children believed that their health was worse than 1 year ago. See Figure 5.5 for a summary of the comparison between the home PN sample, the published norms and the published SBTx data.
## Table 5.6 CHQ-CF87 Self Report Home PN versus Published Normative Data

<table>
<thead>
<tr>
<th>CHQ-CF87 Scale</th>
<th>Home PN Sample Mean (SD) n=4</th>
<th>Published Norms* Mean (SD) n=232</th>
<th>Mean Difference</th>
<th>Effect Size</th>
</tr>
</thead>
<tbody>
<tr>
<td>Physical Functioning</td>
<td>95.4 (5.6) 88.9 100.0</td>
<td>88.8 (14.0)</td>
<td>6.60</td>
<td></td>
</tr>
<tr>
<td>Role/Social Limitations Owing to Physical Function</td>
<td>88.9 (9.1) 77.8 100.0</td>
<td>88.3 (21.0)</td>
<td>0.60</td>
<td></td>
</tr>
<tr>
<td>General Health</td>
<td>33.4 (12.3) 18.8 44.6</td>
<td>66.4 (14.6)</td>
<td>-33.0*</td>
<td>-2.44</td>
</tr>
<tr>
<td>Bodily Pain</td>
<td>77.5 (20.6) 60.0 100.0</td>
<td>74.4 (23.1)</td>
<td>3.1</td>
<td></td>
</tr>
<tr>
<td>Role/Social Limitations Owing to Emotions</td>
<td>94.4 (6.4) 88.9 100.0</td>
<td>85.9 (21.0)</td>
<td>8.5</td>
<td></td>
</tr>
<tr>
<td>Self esteem</td>
<td>70.6 (18.8) 53.8 91.1</td>
<td>81.8 (15.8)</td>
<td>-11.2</td>
<td></td>
</tr>
<tr>
<td>Mental Health</td>
<td>76.2 (16.7) 56.3 92.2</td>
<td>72.7 (16.0)</td>
<td>3.5</td>
<td></td>
</tr>
<tr>
<td>Behavior</td>
<td>74.0 (17.5) 53.5 94.7</td>
<td>76.6 (14.6)</td>
<td>-2.6</td>
<td></td>
</tr>
<tr>
<td>Family Activities</td>
<td>54.4 (10.1) 40 62.5</td>
<td>n/a</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Family Cohesion</td>
<td>72.5 (14.4) 60.0 85.0</td>
<td>n/a</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Global health</td>
<td>45.0 (30.0) 0 60.0</td>
<td>n/a</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Global behavior</td>
<td>78.8 (12.5) 60 85.0</td>
<td>n/a</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Role/Social Limitations Owing to Behaviour</td>
<td>100 (0)</td>
<td>86.5 (21.5)</td>
<td>13.5</td>
<td></td>
</tr>
<tr>
<td>Change in Health Item</td>
<td>Much worse - 0 Somewhat worse - 0 Same - 1 Somewhat better - 1 Much better - 2</td>
<td>n/a</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

*Reference for normative data
*statistically significant p value <0.05
Figure 5.5 Results of CHQ-CF87 Home PN versus Published Normative Data versus Published SBTx Data

*indicates statistical significance (p<0.05) comparing home PN to published norms
^indicates statistical significance (p<0.05) comparing home PN to SBTx Nebraska

5.3.1.2.2 PedsQL4.0™ Self Report Questionnaire

The PedsQL4.0™ Self Report was intended for children greater than 5 years of age and was completed by 6 children on home PN. The results of the comparison between the self reports for all ages from the home PN sample and the published norms can be found in Table 5.7 (See Appendix M for results of the home PN group self assessment separated by age group).

There were significant mean differences found in all scales, the Psychosocial Summary score and the Total QOL score for the PedsQL4.0™ self reports which was consistent with the PedsQL™ proxy report results. The children in the home PN group rated themselves lower than the published norms in Physical, Emotional, Social, and School Functioning. The effect sizes were large in terms of all scales except for the moderate effect size in the social functioning scale. The smallest absolute mean difference in scores was observed in the Physical Functioning scale (10.9 points out of 100 lower in home PN). This was consistent with the comparisons of the home PN sample with published norms using the CHQ-CF87 self-report. In contrast with the emotional/social based results from the CHQ-CF87, there was a statistically significant mean difference of 19 points (out of 100) in the Psychosocial Summary score. In
addition, the Total QOL score was also scored significantly lower by 16 points in the home PN sample compared to the normative sample. See Figure 5.6 for a graph of these results.

Table 5.7 PedsQL4.0TM Self Report Questionnaire Home PN versus Published Normative Data

<table>
<thead>
<tr>
<th>PedsQL4.0™ Self Report Scales</th>
<th>Home PN Sample Mean (SD) Range</th>
<th>Published Norms Mean (SD)</th>
<th>Mean Difference</th>
<th>Effect Size</th>
</tr>
</thead>
<tbody>
<tr>
<td>Physical Functioning Scale</td>
<td>76.6 (9.8) 62.5 - 84.4</td>
<td>87.5 (13.5)</td>
<td>-10.9</td>
<td>-0.924</td>
</tr>
<tr>
<td>Emotional Functioning Scale</td>
<td>62.5 (12.1) 45.0 - 80.0</td>
<td>79.3 (18.2)</td>
<td>-16.8</td>
<td>-1.087</td>
</tr>
<tr>
<td>Social Functioning</td>
<td>69.2 (32.0) 10.0 - 95.0</td>
<td>85.2 (16.8)</td>
<td>-16.0</td>
<td>-0.626</td>
</tr>
<tr>
<td>School Functioning Scale</td>
<td>58.3 (18.9) 35.0 - 75.0</td>
<td>81.1 (16.5)</td>
<td>-22.8</td>
<td>-1.285</td>
</tr>
<tr>
<td>Psychosocial Summary Score</td>
<td>63.3 (13.3) 36.7 - 73.3</td>
<td>81.9 (14.1)</td>
<td>-18.6</td>
<td>-1.357</td>
</tr>
<tr>
<td>Total Score</td>
<td>67.9 (11.4) 45.7 - 77.2</td>
<td>83.8 (12.7)</td>
<td>-15.9</td>
<td>-1.318</td>
</tr>
</tbody>
</table>

*Reference for Normative Data*159

Figure 5.6 Results of PedsQL4.0™ Self Report Home PN versus Published Normative Data

*indicates statistical significance (p<0.05) comparing home PN to published norms
Scales: PF-Physical Functioning, EF-Emotional Functioning, SF-Social Functioning, SC-School Functioning, PSS-Psychosocial Summary Score, TS-Total Score
5.3.2 COMPARISON OF THE HOME PN GENERIC QOL SURVEY RESULTS TO PUBLISHED SMALL BOWEL TRANSPLANT DATA

5.3.2.1 Proxy Reports

5.3.2.1.1 Child Health Questionnaire Parent Form 50 (CHQ-PF50)

The results from the CHQ-PF50 proxy form (for ages 5 years and up) in the home PN sample was also compared to published SBTx data\textsuperscript{18,83}. Five proxy respondents completed the form and were compared to two samples of SBTx proxy respondents (n=22 and n=33). See Table 5.8 for a summary of results between the home PN sample and the Nebraska and UCLA SBTx samples.

There were no statistically significant results between the home PN group and the two published SBTx samples. Differences may have gone undetected as a result of the small sample sizes in all groups, especially in the home PN sample\textsuperscript{18}. The home PN group had scored lower on the Physical Functioning scale compared to the SBTx groups however; there was a very wide range of scores (17 to 94 out of 100). The same occurred in the Bodily Pain scale. In terms of how their physical function impacted schoolwork, activities and time with friends, the home PN scores were between 11 and 17 points lower than the SBTx results. Overall assessment of General Health was between 6 and 10 points lower in the home PN group compared to the SBTx groups. Although these scores were not significantly different, it was noted that all 3 samples rated themselves less than 50 (out of a possible 100). Parental Impact appeared to be similar in all of the groups however there was a smaller mean difference in the impact on Parental Time compared to the impact on Parental Emotions. The Family Cohesion factor in the home PN was rated slightly higher than the SBTx populations and the range of the home PN scores was quite narrow (85 to 100). Family Activities appeared lower in the home PN sample according to mean differences, but the SD was large (38.8) in the home PN sample whereas the SD in the other two SBTx groups were almost half of that. The Self Esteem, Mental Health and Behaviour domains were similar. See Figure 5.3 for a graphical representation comparing the Home PN group to the normative population and the two SBTx samples.
Table 5.8 CHQ-PF50 Proxy Report Home PN versus Published SBTx Data

<table>
<thead>
<tr>
<th>CHQ-PF50 Scales</th>
<th>Home PN Sample</th>
<th>Published SBTx Sample\a (Sudan et al., Nebraska)</th>
<th>Published SBTx Sample\b (Ngo et al., UCLA)</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>Mean (SD)</td>
<td>Mean (SD)</td>
<td>Mean (SD)</td>
</tr>
<tr>
<td></td>
<td>n=5</td>
<td>N=22</td>
<td>n=33</td>
</tr>
<tr>
<td>Physical Functioning</td>
<td>72.2 (31.7)</td>
<td>80.1 (20.6)</td>
<td>85.1 (24.7)</td>
</tr>
<tr>
<td></td>
<td>16.7 ± 94.4</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Role/Social Limitations Owing to Physical Function</td>
<td>60.0 (38.4)</td>
<td>74.3 (31.1)</td>
<td>77.4 (29.4)</td>
</tr>
<tr>
<td></td>
<td>0 ± 100.0</td>
<td></td>
<td></td>
</tr>
<tr>
<td>General Health</td>
<td>34.2 (9.9)</td>
<td>42.6 (14.4)</td>
<td>45.4 (18.5)</td>
</tr>
<tr>
<td></td>
<td>26.7 ± 51.7</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Bodily Pain</td>
<td>64.0 (35.1)</td>
<td>75 (20.9)</td>
<td>77.8 (18.0)</td>
</tr>
<tr>
<td></td>
<td>10.0 ± 100.0</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Role/Social Limitations Owing to Emotions/Behavior</td>
<td>60.0 (43.5)</td>
<td>81.5 (23.8)</td>
<td>66.0 (34.1)</td>
</tr>
<tr>
<td></td>
<td>0 ± 100.0</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Self esteem</td>
<td>68.2 (34.3)</td>
<td>71.2 (17.6)</td>
<td>76.1 (23.2)</td>
</tr>
<tr>
<td></td>
<td>8.3 ± 95.0</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Mental Health</td>
<td>79.0 (19.2)</td>
<td>75.4 (9.4)</td>
<td>75.8 (15.1)</td>
</tr>
<tr>
<td></td>
<td>45.0 ± 90.0</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Behavior</td>
<td>73.0 (17.0)</td>
<td>71 (14.6)</td>
<td>61.4 (17.9)</td>
</tr>
<tr>
<td></td>
<td>51.7 ± 97.5</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Family Activities</td>
<td>54.2 (38.8)</td>
<td>69.6 (19.8)</td>
<td>64.1 (22.6)</td>
</tr>
<tr>
<td></td>
<td>0 ± 95.8</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Family Cohesion</td>
<td>88.8 (7.5)\c</td>
<td>74 (14.9)</td>
<td>81.2 (15.4)</td>
</tr>
<tr>
<td></td>
<td>85.0 ± 95.0</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Parental impact on emotion</td>
<td>56.7 (35.1)</td>
<td>61.8 (26.8)</td>
<td>54.1 (29.6)</td>
</tr>
<tr>
<td></td>
<td>8.3 ± 91.7</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Parental impact on time</td>
<td>60.0 (32.0)</td>
<td>74.1 (24.7)</td>
<td>66.7 (32.1)</td>
</tr>
<tr>
<td></td>
<td>22.2 ± 100.0</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Global health</td>
<td>60.0 (0)</td>
<td>73.5 (23.1)</td>
<td>-</td>
</tr>
<tr>
<td>Global behavior</td>
<td>78.0 (17.5)</td>
<td>72.3 (24.7)</td>
<td>-</td>
</tr>
<tr>
<td></td>
<td>60.0 ± 100.0</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Summary ß Physical</td>
<td>32.2 (16.5)</td>
<td>40.6 (14.8)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>4.96 ± 49.2</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Summary ß Psychosocial</td>
<td>45.5 (15.8)</td>
<td>41.1 (14.6)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>20.2 ± 59.8</td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

\aReference for small bowel transplant comparative data\83
\bReference for intestinal transplant data\83
\cFamily cohesion scale incomplete (Home PN n=4)

5.3.2.1.2 PedsQL4.0™ Proxy Report Questionnaires

The comparison of the home PN sample to the SBTx sample was done using the abstract presented by Ngo et al at the 2009 International Small Bowel Transplant Symposium\83, the PedsQL was not utilized in the study by Sudan et al. See Table 5.9 for a summary of results between the home PN sample and the SBTx sample.
Table 5.9 PedsQL4.0™ Proxy Report Questionnaire Home PN versus Published SBTx Data

<table>
<thead>
<tr>
<th>PedsQL4.0™ Proxy Report Scales</th>
<th>Home PN Sample Mean (SD)</th>
<th>Published SBTx Samplea (Ngo et al., UCLA) Mean (SD)</th>
<th>Mean Difference</th>
</tr>
</thead>
<tbody>
<tr>
<td>Physical Functioning Scale</td>
<td>59.4 (21.8) 21.9 - 87.5</td>
<td>66.1 (22.8)</td>
<td>-6.7</td>
</tr>
<tr>
<td>Emotional Functioning Scale</td>
<td>62.8 (16.6) 45.0 - 100.0</td>
<td>72.3 (18.9)</td>
<td>-9.5</td>
</tr>
<tr>
<td>Social Functioning Scale</td>
<td>64.4 (28.4) 10.0 - 100.0</td>
<td>63.0 (20.0)</td>
<td>1.4</td>
</tr>
<tr>
<td>School Functioning Scale</td>
<td>56.0 (28.4)b 20.0 - 90.0</td>
<td>46.1 (22.6)</td>
<td>9.9</td>
</tr>
<tr>
<td>Psychosocial Summary Score</td>
<td>61.3 (19.2) 31.7 - 90.0</td>
<td>65.3 (16.9)</td>
<td>-4.0</td>
</tr>
<tr>
<td>Total Score</td>
<td>60.3 (18.3) 39.1 - 88.0</td>
<td>64.2 (17.6)</td>
<td>-3.9</td>
</tr>
</tbody>
</table>

*aReference for comparative data83*

The proxy report was completed by 9 respondents from the home PN group (for ages 2 years and older) and was compared to 33 proxy respondents from the published SBTx population. In this study, there were non-significant p values related to the mean differences between the home PN sample and the SBTx sample. The absolute values of the scale and summary scores were similar with mean differences ranging between 1.4 and 9.9 points on a scale of 0 to 100. The scales that were closest to a 10 point difference included the Emotional and School Functioning scales. The Emotional Functioning scale was associated with how often the child was angry, sad, worried or had difficulty sleeping. The SD was similar in both groups indicating a similar range of scores on that scale. The School Functioning score for the home PN group was approximately 10 points higher and related to the impact of chronic illness on class participation, school attendance and keeping up with peers. The SD was larger in the home PN sample suggesting a wider range of scores compared to the SBTx sample. Overall, the results for both groups were similar. See Figure 5.4 for a graph of the comparison of the PedsQL4.0™ Proxy Report between the home PN sample, the SBTx UCLA sample and the published normative data.

5.3.2.2 Child Self Report

5.3.2.2.1 Child Health Questionnaire Child Form 87 (CHQ-CF87)

The intestinal transplant recipients that were used in comparison for the CHQ-CF87 results were published in the article by Sudan et al.18 as Ngo et al. solely investigated parent proxy. Four
children (greater than 10 years of age) on home PN completed the survey and were compared to 22 children post SBTx. Results can be found in Table 5.10 comparing the home PN population at SickKids to the published data of intestinal recipients from Nebraska.

**Table 5.10** CHQ-CF87 Self Report Home PN versus Published SBTx Data

<table>
<thead>
<tr>
<th>CHQ-CF87 Scales</th>
<th>Home PN Sample</th>
<th>SBTx Sample* (Sudan et al., Nebraska)</th>
<th>Mean Difference</th>
<th>Effect Size</th>
</tr>
</thead>
<tbody>
<tr>
<td>Physical Functioning</td>
<td>95.4 (5.6)</td>
<td>87.2 (14.6)</td>
<td>8.2</td>
<td>-</td>
</tr>
<tr>
<td>Role/Social Limitations Owing to Physical Function</td>
<td>88.9 (9.1)</td>
<td>87.3 (19.3)</td>
<td>1.6</td>
<td>-</td>
</tr>
<tr>
<td>General Health</td>
<td>33.4 (12.3)</td>
<td>61.8 (18.5)</td>
<td>-28.4*</td>
<td>-1.81</td>
</tr>
<tr>
<td>Bodily Pain</td>
<td>77.5 (20.6)</td>
<td>79.1 (25.6)</td>
<td>-1.6</td>
<td>-</td>
</tr>
<tr>
<td>Role/Social Limitations Owing to Emotions</td>
<td>94.4 (6.4)</td>
<td>84.7 (19.1)</td>
<td>9.7</td>
<td>-</td>
</tr>
<tr>
<td>Self esteem</td>
<td>70.6 (18.8)</td>
<td>78.5 (14.3)</td>
<td>-7.9</td>
<td>-</td>
</tr>
<tr>
<td>Mental Health</td>
<td>76.2 (16.7)</td>
<td>78.2 (8.0)</td>
<td>-2.0</td>
<td>-</td>
</tr>
<tr>
<td>Behavior</td>
<td>74.0 (17.5)</td>
<td>74 (12.2)</td>
<td>0</td>
<td>-</td>
</tr>
<tr>
<td>Family Activities</td>
<td>54.4 (10.1)</td>
<td>71.3 (22.0)</td>
<td>-16.9</td>
<td>-</td>
</tr>
<tr>
<td>Family Cohesion</td>
<td>72.5 (14.4)</td>
<td>76.9 (18.6)</td>
<td>-4.4</td>
<td>-</td>
</tr>
<tr>
<td>Global health</td>
<td>45.0 (30.0)</td>
<td>79.3 (22.4)</td>
<td>-34.3*</td>
<td>-1.30</td>
</tr>
<tr>
<td>Global behavior</td>
<td>78.8 (12.5)</td>
<td>73.1 (21.2)</td>
<td>5.7</td>
<td>-</td>
</tr>
<tr>
<td>Role/Social Limitations Owing to Behaviour</td>
<td>100 (0)</td>
<td>n/a</td>
<td>-</td>
<td>-</td>
</tr>
</tbody>
</table>

*Reference for intestinal transplant data*

*statistically significant at p value <0.05

Upon comparison of the two populations, there was a statistically significant mean difference (p=0.008) on the scale of **General Health Perceptions** demonstrating a large effect size (-1.81). The home PN sample scored the 12-item General Health scale a mean of 28 points less than the published SBTx population, representing concerns about overall health, both current and future. The home PN group also scored the **Global Health** item statistically lower with a large effect size (p=0.014, effect size= -1.30). The Global Health item was scored a mean of 34 points lower in the home PN population compared to the published SBTx group. This single item question
asked the children to compare their health to other children of their age and was scored very low in the home PN population (mean = 45 out of 100) with a slightly higher SD than the SBTx sample. This item was not included in the normative evaluation by Landgraff preventing further comparison with ratings from healthy children. Other domains appeared to be similar between groups in terms of mean ratings, including Physical Functioning and Self Esteem.

Although not statistically significant, the home PN population rated the Family Activities scale 17 points lower than the intestinal transplant patients. This scale related to the child’s evaluation of their health and its role in interrupting and impacting activities within their family. See Figure 5.5 for a summary of the comparison between the home PN sample, the SBTx sample from Nebraska and the published norms.

5.4 Secondary Quantitative Analysis

5.4.1 COMPARISON OF HOME PN SAMPLE VERSUS INSTITUTIONAL SBTX SAMPLE

A comparative analysis of scores from the battery assessment between the home PN sample versus the SickKids SBTx sample was completed. The scales included were the ITQOL, CHQ-PF50, PedsQL4.0™ Proxy, Self and Family Impact Modules and the Visual Analogue Scales (VAS). The comparative analyses were done utilizing non-parametric statistics however, mean differences were presented in order to facilitate descriptive analysis of survey scores. The Functional Status IIR was compared to the institutional SBTx and a published well and published chronically ill sample. An analysis was not done for the CHQ-CF87 as there were no children from the SBTx sample old enough (10 to 18 years) to complete the questionnaire.

5.4.1.1 Infant Toddler Quality of Life (ITQOL) Questionnaire

There were 10 proxy assessments of children in the home PN group available for comparison to 2 proxy assessments in the SBTx group. The results for the non-parametric analysis can be found in Table 5.11. There were no statistically significant differences demonstrated between the two comparison groups although the analysis was considerably limited due to the very small sample size in the SBTx group. The 2 proxy scores in the SBTx group differed from each other by greater than 30 points (out of a total of 100) in many scales including the Global Health item,
Physical Functioning, the Global Behaviour item, Parental Impact on Emotion and Family Cohesion. Consequently, the interpretation of the summary statistics mean and median must take that into consideration. Both groups had low median results for the Global Health item and General Health Perceptions and rated their children similarly high in the scales Growth and Development and Temperament and Moods. They also had very similar median ratings and IQR for Parental Impact on Time as well as Family Cohesion. In the SBTx sample for the item: Change in Health over the past year, these proxies both felt that the children were much better. In the home PN sample, 1 proxy felt that the child’s health was much worse, 1 felt that it was the same, 3 felt that it was somewhat better and 2 felt that the child’s health was much better.

Table 5.11 ITQOL Proxy Report Questionnaire Home PN versus Institutional SBTx

<table>
<thead>
<tr>
<th>ITQOL Scales</th>
<th>Home PN Sample Median (IQR) n=10</th>
<th>SBTx Sample Median (IQR) n=2</th>
<th>Wilcoxon Rank P value</th>
<th>Mean Difference</th>
</tr>
</thead>
<tbody>
<tr>
<td>Global Health Item</td>
<td>60.0 (30.0 I 60.0)</td>
<td>57.5 (30.0 I 85.0)</td>
<td>0.827</td>
<td>-7.50</td>
</tr>
<tr>
<td>Physical Functioning</td>
<td>73.3 (61.7 I 90.0)</td>
<td>90.0 (57.5 I 95.0)</td>
<td>0.518</td>
<td>-14.17</td>
</tr>
<tr>
<td>Growth and Development</td>
<td>75.0 (65.0 I 80.6)</td>
<td>76.3 (80.0 I 100.0)</td>
<td>0.916</td>
<td>-2.75</td>
</tr>
<tr>
<td>Bodily Pain</td>
<td>70.8 (66.7 I 75.0)</td>
<td>54.2 (41.7 I 66.7)</td>
<td>0.212</td>
<td>15.83</td>
</tr>
<tr>
<td>Temperament and Moods</td>
<td>77.8 (70.8 I 80.6)</td>
<td>78.5 (69.4 I 87.5)</td>
<td>0.916</td>
<td>-1.11</td>
</tr>
<tr>
<td>General Behaviour</td>
<td>77.1 (62.5 I 93.8)</td>
<td>80.2 (68.8 I 91.7)</td>
<td>1.000</td>
<td>-6.70</td>
</tr>
<tr>
<td>Global Behaviour Item</td>
<td>85.0 (60.0 I 100.0)</td>
<td>80.0 (60.0 I 100.0)</td>
<td>1.000</td>
<td>-2.14</td>
</tr>
<tr>
<td>Getting Along</td>
<td>76.7 (65.0 I 78.6)</td>
<td>60.8 (60.0 I 61.7)</td>
<td>0.092</td>
<td>13.01</td>
</tr>
<tr>
<td>General Health Perceptions</td>
<td>30.7 (20.5 I 47.7)</td>
<td>31.8 (18.2 I 45.5)</td>
<td>0.833</td>
<td>3.41</td>
</tr>
<tr>
<td>Parental Impact: Emotion</td>
<td>76.8 (53.6 I 85.7)</td>
<td>62.5 (39.3 I 85.7)</td>
<td>0.753</td>
<td>9.30</td>
</tr>
<tr>
<td>Parental Impact: Time</td>
<td>74.4 (66.7 I 85.7)</td>
<td>76.2 (66.7 I 85.7)</td>
<td>1.000</td>
<td>-1.19</td>
</tr>
<tr>
<td>Family Cohesion</td>
<td>72.5 (60.0 I 100.0)</td>
<td>80.0 (60.0 I 100.0)</td>
<td>0.909</td>
<td>-3.00</td>
</tr>
</tbody>
</table>

*a* Physical Functioning Scale analysis utilized only complete scales (Home PN n=4)

*b* Applicable for children >1 year only (Home PN n=7)

5.4.1.2 Child Health Questionnaire Parent Form 50 (CHQ-PF50)

There were 5 proxy assessments of children in the home PN group for comparison to 4 proxy assessments in the SBTx group using the CHQ-PF50. The results for the non-parametric analysis can be found in Table 5.12. There were no statistically significant differences demonstrated between the two comparison groups although small sample sizes limited this analysis. The absolute values were reviewed and the mean differences were relatively small with
similar scores appearing in both groups. Both the home PN and the SBTx groups scored the lowest median scores on the **General Health** scale, 30.8 and 35.0 respectively (out of 100). They also scored similar low median scores on the **Global Health** item. Similar high scores were rated for **Bodily Pain, Self Esteem, Mental Health, Behaviour**, and also on the **Physical** and **Psychosocial Summary** scales. Two scales had overall mean differences of approximately 13; the **Role/Social Limitations Owing to Emotions & Behaviour** scale was 13 mean points higher for home PN and the **Family Activities** scale was 13 points lower in home PN. The range for the SBTx group in these two scales was large (0 to 100 and 33 to 100 respectively). The categorical item, **Change in Health** over the past year, demonstrated that neither proxies from the home PN nor the SBTx sample felt that their child’s health was much worse than the year before. The majority of respondents in both groups felt that their child’s health over the past year ranged from staying the same to much better.

**Table 5.12 CHQ-PF50 Proxy Report Home PN versus Institutional SBTx**

<table>
<thead>
<tr>
<th>CHQ-PF50 Scales</th>
<th>Home PN Sample Median (IQR) n=5</th>
<th>SBTx Sample Median (IQR) n=4</th>
<th>Wilcoxon Rank P value</th>
<th>Mean Difference</th>
</tr>
</thead>
<tbody>
<tr>
<td>Physical Functioning</td>
<td>83.3 (77.8 – 88.9)</td>
<td>69.4 (52.8 – 83.3)</td>
<td>0.636</td>
<td>4.17</td>
</tr>
<tr>
<td>Role/Social limitations Owing</td>
<td>66.7 (50.0 – 83.3)</td>
<td>83.3 (41.7 – 100.0)</td>
<td>0.630</td>
<td>-10.83</td>
</tr>
<tr>
<td>to Physical Function</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>General Health</td>
<td>30.8 (30.8 – 30.8)</td>
<td>35.0 (32.9 – 43.3)</td>
<td>0.279</td>
<td>-3.96</td>
</tr>
<tr>
<td>Bodily Pain</td>
<td>80.0 (50.0 – 80.0)</td>
<td>80.0 (60.0 – 100.0)</td>
<td>0.547</td>
<td>-16.0</td>
</tr>
<tr>
<td>Role/Social Limitations Owing</td>
<td>66.7 (33.3 – 100.0)</td>
<td>44.4 (11.1 – 83.3)</td>
<td>0.716</td>
<td>12.78</td>
</tr>
<tr>
<td>to Emotions &amp; Behaviour</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Self esteem</td>
<td>79.2 (75.0 – 83.3)</td>
<td>71.7 (52.9 – 89.6)</td>
<td>1.000</td>
<td>-3.08</td>
</tr>
<tr>
<td>Mental Health</td>
<td>85.0 (85.0 – 90.0)</td>
<td>80.0 (77.5 – 82.5)</td>
<td>0.291</td>
<td>-1.00</td>
</tr>
<tr>
<td>Behavior</td>
<td>75.0 (64.2 – 76.7)</td>
<td>72.5 (57.5 – 87.1)</td>
<td>1.000</td>
<td>0.71</td>
</tr>
<tr>
<td>Family Activities</td>
<td>45.8 (41.7 – 87.5)</td>
<td>60.4 (45.8 – 79.2)</td>
<td>0.812</td>
<td>-8.33</td>
</tr>
<tr>
<td>Family Cohesion</td>
<td>85.0 (85.0 – 92.5)*</td>
<td>72.5 (60.0 – 92.5)</td>
<td>0.461</td>
<td>12.5</td>
</tr>
<tr>
<td>Parental impact on emotion</td>
<td>66.7 (33.3 – 83.3)</td>
<td>58.3 (25.0 – 91.7)</td>
<td>0.904</td>
<td>-1.67</td>
</tr>
<tr>
<td>Parental impact on time</td>
<td>66.7 (33.3 – 77.8)</td>
<td>50.0 (27.8 – 83.3)</td>
<td>0.904</td>
<td>-4.44</td>
</tr>
<tr>
<td>Global health</td>
<td>60.0 (60.0 – 60.0)</td>
<td>60.0 (60.0 – 72.5)</td>
<td>0.397</td>
<td>-6.25</td>
</tr>
<tr>
<td>Global behavior</td>
<td>85.0 (60.0 – 85.0)</td>
<td>72.5 (45.0 – 85.0)</td>
<td>0.533</td>
<td>13.0</td>
</tr>
<tr>
<td>Change in Health Item</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Much worse † 0</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Worse † 1</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Same † 2</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Somewhat better † 1</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Much better † 1</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Much worse † 0</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Worse † 0</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Same † 1</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Somewhat better † 1</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Much better † 2</td>
<td></td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Summary † Physical</td>
<td>35.0 (32.0 – 39.6)</td>
<td>35.7 (30.2 – 41.1)</td>
<td>1.000</td>
<td>-3.54</td>
</tr>
<tr>
<td>Summary † Psychosocial</td>
<td>47.5 (42.7 – 57.4)</td>
<td>44.1 (28.9 – 58.6)</td>
<td>1.000</td>
<td>1.81</td>
</tr>
</tbody>
</table>

*based on n=4 for home PN
5.4.1.3 PedsQL4.0™ Proxy-Report Scores

The PedsQL4.0™ Proxy Rating QOL tool was administered accordingly in age-appropriate formats. The proxy results, separated by age, for both the home PN and the SBTx groups are presented in Appendix L. Due to small sample sizes in each category the results for each tool were summarized to include all ages and a non-parametric analysis was conducted on the summary data, see Table 5.13. The total sample for the home PN group that completed the proxy QOL assessment tool was 9 (4 toddler reports, 1 young child report, 2 child reports and 2 adolescent reports) and 6 in the SBTx group (2 toddler reports, 3 young child reports and 1 child report). There were no adolescent proxy-reports in the SBTx group. Utilizing the Wilcoxon Rank test, there were no statistically significant differences between the 2 groups neither on the individual scales, nor on the Psychosocial Summary and Total Scores. The absolute mean differences were all less than 10 points for each scale. The ranges for the scales were slightly smaller for the SBTx group indicating less variability with the scoring. Mean and median scale scores were lower, albeit non-significantly, for all scales in the home PN group with the exception of the School Functioning which was ranking slightly higher in the home PN group.

Table 5.13 PedsQL4.0™ Proxy Report Questionnaire Home PN versus Institutional SBTx

<table>
<thead>
<tr>
<th>PedsQL4.0™ Proxy Report Scales</th>
<th>Home PN Sample</th>
<th>SBTx Sample</th>
<th>Wilcoxon Rank P value</th>
<th>Mean Difference</th>
</tr>
</thead>
<tbody>
<tr>
<td>Physical Functioning Scale</td>
<td>53.1 (53.1 ± 78.1)</td>
<td>68.3 (56.3 ± 71.9)</td>
<td>0.6852</td>
<td>-4.99</td>
</tr>
<tr>
<td>Emotional Functioning Scale</td>
<td>60.0 (50.0 ± 65.0)</td>
<td>75.0 (65.0 ± 75.0)</td>
<td>0.1572</td>
<td>-8.06</td>
</tr>
<tr>
<td>Social Functioning Scale</td>
<td>60.0 (50.0 ± 90.0)</td>
<td>75.0 (75.0 ± 80.0)</td>
<td>0.6428</td>
<td>-7.22</td>
</tr>
<tr>
<td>School Functioning Scale</td>
<td>66.7 (30.0 ± 80.0)</td>
<td>55.0 (35.0 ± 65.0)</td>
<td>0.5214</td>
<td>5.95</td>
</tr>
<tr>
<td>Psychosocial Summary Score</td>
<td>57.7 (53.1 ± 73.1)</td>
<td>70.8 (53.3 ± 75.0)</td>
<td>0.7287</td>
<td>-5.38</td>
</tr>
<tr>
<td>Total Score</td>
<td>61.1 (44.0 ± 70.2)</td>
<td>67.8 (58.7 ± 75.0)</td>
<td>0.4556</td>
<td>-5.43</td>
</tr>
</tbody>
</table>

*School Functioning scale in the toddler form is only appropriate for those in daycare or school, thus the results in the home PN group are based on n=8

bSchool Functioning scale in the toddler form is only appropriate for those in daycare or school, thus the results in the SBTx are based on n=4

5.4.1.4 PedsQL4.0™ Self Report Scores

The PedsQL4.0™ Self Scoring Quality of Life tool was administered in its age-appropriate formats. The results for both the home PN and the SBTx groups are presented for each age-specific tool in Appendix M. Due to small sample sizes in each category the results for each tool were summarized to include all ages and non-parametric analyses were done on the
summary data. See Table 5.14. Following the combination of all age groups, there were 6 respondents in the home PN group (1 young child report, 2 child reports and 3 adolescent reports) and 3 respondents in the SBTx group (2 young child reports and 1 child report) that were available for comparison. There were no adolescent reports in the SBTx group. The age differences of respondents between the groups were taken into consideration for interpretation. Non-parametric analysis, utilizing the Wilcoxon Rank test, did not reveal any significant differences between the two groups. Large mean differences were found for the Social and School Functioning scales (33 and 31, respectively) and also for the Psychosocial Summary score and the total score (21 and 17, respectively). The Social Functioning scale asked children how they related to others in terms of teasing, getting along with others and keeping up with friends and peers. The home PN group had a wide range of scores in this domain (10 to 95 out of 100), however the median was 80 with an IQR of 60-90. The 3 SBTx children scored a large spectrum of responses on the Social Functioning scale 0, 40 and 70. The Social and School functioning scores were the most variable for the SBTx group with the widest ranges. In terms of pain, 2 young children in the SBTx group answered ‘sometimes’ when asked if they ever have pain and specified ‘in their tummy’.

Table 5.14 PedsQL4.0™ Self Report Questionnaires Home PN versus Institutional SBTx

<table>
<thead>
<tr>
<th>PedsQL4.0™ Self Report Scales</th>
<th>Home PN Sample Median (IQR) n=6</th>
<th>SBTx Sample Median (IQR) n=3</th>
<th>Wilcoxon Rank P value</th>
<th>Mean Difference</th>
</tr>
</thead>
<tbody>
<tr>
<td>Physical Functioning Scale</td>
<td>81.3 (65.6 – 84.4)</td>
<td>68.8 (56.3 – 75.0)</td>
<td>0.275</td>
<td>9.90</td>
</tr>
<tr>
<td>Emotional Functioning Scale</td>
<td>62.5 (55.0 – 70.0)</td>
<td>55.0 (50.0 – 87.5)</td>
<td>1.000</td>
<td>-1.67</td>
</tr>
<tr>
<td>Social Functioning Scale</td>
<td>80.0 (60.0 – 90.0)</td>
<td>40.0 (0.0 – 70.0)</td>
<td>0.229</td>
<td>32.50</td>
</tr>
<tr>
<td>School Functioning Scale</td>
<td>65.0 (35.0 – 75.0)</td>
<td>20.0 (12.5 – 50.0)</td>
<td>0.129</td>
<td>30.83</td>
</tr>
<tr>
<td>Psychosocial Summary Score</td>
<td>67.5 (66.7 – 68.3)</td>
<td>46.7 (30.8 – 48.3)</td>
<td>0.129</td>
<td>21.41</td>
</tr>
<tr>
<td>Total Score</td>
<td>72.3 (67.4 – 72.8)</td>
<td>51.1 (45.2 – 56.5)</td>
<td>0.130</td>
<td>16.99</td>
</tr>
</tbody>
</table>

5.4.1.5 PedsQL™ Family Impact Module Scores

The PedsQL™ Family Impact Module was used to measure the functioning of the proxy as a result of their child’s health over the past month and is used in children from birth to 18 years. There was no normative data available for comparison of the Family Impact Module to these samples. The results of the scores for the home PN and SBTx participants can be found in Table 5.15 using non-parametric analysis for comparison. There were 15 respondents in the home PN group and 6 respondents in the SBTx group. There were no significant differences between the
two groups. Most scores were similar between the 2 groups with less than 10 points mean difference. On a scoring scale of 0 to 100, almost all scales demonstrated mean scores less than 65, excluding the family relationships scale for the home PN group and the cognitive functioning scale for both groups. The median parent QOL summary scores, the median family functioning summary score and the total scores between home PN and SBTx were within 5 points in both the mean and median scores. The lowest median scale scores were related to the worry scale and the daily activities scale. The worry scale asked the proxy to reflect on the amount of worry they experience regarding whether medical treatments were working, their side effects, the impact on other family members, reactions from others and their child’s future. The daily activities scale related to energy and time available for household tasks and the impact of the child’s health on the time taken for family activities.

Table 5.15 PedsQL4.0™ Family Impact Module Home PN versus Institutional SBTx

<table>
<thead>
<tr>
<th>PedsQL4.0™ Family Impact Module Scales</th>
<th>Home PN Sample Median (IQR) n=15</th>
<th>SBTx Sample Median (IQR) n=6</th>
<th>Wilcoxon Rank P value</th>
<th>Mean Difference</th>
</tr>
</thead>
<tbody>
<tr>
<td>Physical Functioning Scale</td>
<td>58.3 (37.5–75.0)</td>
<td>58.3 (16.7–66.7)</td>
<td>0.443</td>
<td>10.56</td>
</tr>
<tr>
<td>Emotional Functioning Scale</td>
<td>50.0 (45.0–70.0)</td>
<td>55.0 (25.0–65.0)</td>
<td>0.847</td>
<td>8.33</td>
</tr>
<tr>
<td>Social Functioning Scale</td>
<td>50.0 (25.0–81.3)</td>
<td>68.8 (50.0–75.0)</td>
<td>0.785</td>
<td>-8.96</td>
</tr>
<tr>
<td>Cognitive Functioning Scale</td>
<td>75.0 (60.0–85.0)</td>
<td>75.0 (70.0–75.0)</td>
<td>0.845</td>
<td>5.00</td>
</tr>
<tr>
<td>Communication Scale</td>
<td>50.0 (33.3–66.7)</td>
<td>66.7 (58.3–75.0)</td>
<td>0.268</td>
<td>-8.89</td>
</tr>
<tr>
<td>Worry Scale</td>
<td>45.0 (35.0–50.0)</td>
<td>45.0 (35.0–50.0)</td>
<td>0.786</td>
<td>-6.00</td>
</tr>
<tr>
<td>Daily Activities Scale</td>
<td>41.7 (25.0–66.7)</td>
<td>45.8 (41.7–58.3)</td>
<td>0.443</td>
<td>-10.28</td>
</tr>
<tr>
<td>Family Relationships Scale</td>
<td>70.0 (60.0–100.0)</td>
<td>72.5 (50.0–75.0)</td>
<td>0.642</td>
<td>10.33</td>
</tr>
<tr>
<td>Parent QOL Summary Score</td>
<td>67.5 (40.0–73.8)</td>
<td>62.8 (32.5–70.0)</td>
<td>0.491</td>
<td>4.82</td>
</tr>
<tr>
<td>Family Functioning Summary Score</td>
<td>62.5 (46.9–75.0)</td>
<td>62.5 (43.8–68.8)</td>
<td>0.758</td>
<td>2.60</td>
</tr>
<tr>
<td>Total Score</td>
<td>65.3 (40.3–68.8)</td>
<td>61.9 (36.8–70.1)</td>
<td>0.848</td>
<td>1.70</td>
</tr>
</tbody>
</table>

5.4.1.6 Functional Status IIR

A comparative analysis was conducted for the Functional Status IIR 14-item scale scores in home PN versus the institutional SBTx participants. In addition, the home PN population was compared to published ill and well samples. Results from this analysis can be found in Table 5.16. The scale scores range from 0 to 100 with a higher score indicating higher functional status. In the SickKids samples, there were 2 respondents who did not complete all 14 items. The recommendations by the author were to eliminate these from the analysis. However due to the small sample size, the denominator for the final score calculation for the two surveys was adjusted to reflect the number of items missed and surveys were included in the analysis.
The home PN group did not demonstrate a significant mean difference in scores when compared to both the published chronically ill sample and the institutional SBTx sample. The absolute values for the mean scores and the SD for all three groups were similar. There was a highly significant difference found (p<0.001) in the mean total scores with a large effect size of -1.07 when comparing the home PN sample to the published well children sample (84.1±13.5 versus 96.1±8.2, respectively). See Figure 5.6 for a graphical representation of these results.

**Table 5.16** Functional Status IIIR Proxy Report Home PN versus Institutional SBTx versus Published Data of Ill and Well Children

<table>
<thead>
<tr>
<th>COMPARISON GROUP</th>
<th>Home PN Sample</th>
<th>Published Ill Sample</th>
<th>Published Well Sample</th>
<th>Institutional SBTx Sample</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td>n=15</td>
<td>n=456</td>
<td>n=276</td>
<td>n=6</td>
</tr>
<tr>
<td></td>
<td>Mean (SD) = 84.1 (13.5)</td>
<td>Mean (SD) = 86.8 (15.7)</td>
<td>Mean (SD) = 96.1 (8.2)</td>
<td>Mean (SD)= 82.1 (13.7)</td>
</tr>
<tr>
<td></td>
<td>Range: 58.3 – 100</td>
<td></td>
<td></td>
<td>Range: 64.3 – 100</td>
</tr>
<tr>
<td>Mean Difference</td>
<td>-2.70</td>
<td>-12.0</td>
<td>1.93</td>
<td></td>
</tr>
<tr>
<td>P Value*</td>
<td>0.511</td>
<td>&lt;0.001</td>
<td>0.772</td>
<td></td>
</tr>
<tr>
<td>Effect Size</td>
<td>-1.07</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

*a all p values were calculated using pooled results as all determined to have equal variances

**Figure 5.7** Functional Status IIIR Home PN versus Institutional SBTx, Published Well and Published Chronically Ill Data

84.1 82.1 86.8 96.1

*indicates statistical significance (p<0.05) comparing home PN to published norms

5.4.1.7 Proxy Rating of Visual Analogue Scales

The Visual Analogue Scale (VAS) results pertaining to the 3 measured attributes: perceived QOL, pain and overall health, were assessed by comparing the home PN sample with the institutional SBTx population for both proxy and child assessment. The VAS results were a measurement of a continuum on a straight line from 0 to 10 cm indicating worst to best results.
Therefore, a higher score was equivalent to a better result (best QOL, no pain and best overall health).

The results and comparative analysis of the proxy reported VAS scores using the Wilcoxon Rank Sum test are in Table 5.17. There were 15 respondents from the home PN group and 6 respondents in the SBTx group. The home PN group had lower mean and median scores for all 3 domains. The analysis demonstrated significant p values < 0.05 for the scales Overall Health and Pain with mean differences of -2.7 and -2.0, respectively. There was no significant difference between the two groups for the proxy measure of overall QOL despite a mean difference of -1.9, with the home PN group having the lower score.

Table 5.17 VAS Proxy Report Home PN versus Institutional SBTx

<table>
<thead>
<tr>
<th>VAS Scale – Proxy Report</th>
<th>Home PN Sample Median (IQR)</th>
<th>SBTx Sample Median (IQR)</th>
<th>Wilcoxon Rank P Value</th>
<th>Mean Difference</th>
</tr>
</thead>
<tbody>
<tr>
<td>Quality of Life</td>
<td>5.6 (5.0 ÷ 7.3)</td>
<td>8.9 (7.2 ÷ 9.2)</td>
<td>0.2195</td>
<td>-1.9</td>
</tr>
<tr>
<td>Pain</td>
<td>7.5 (5.7 ÷ 8.3)</td>
<td>9.4 (8.1 ÷ 9.7)</td>
<td><strong>0.0254</strong></td>
<td>-2.0</td>
</tr>
<tr>
<td>Overall Health</td>
<td>6.2 (4.6 ÷ 7.4)</td>
<td>8.8 (7.8 ÷ 9.3)</td>
<td><strong>0.0102</strong></td>
<td>-2.7</td>
</tr>
</tbody>
</table>

aHome PN sample size for Quality of Life assessment n=13
bHome PN sample size for Overall Health assessment n=14

A categorical assessment of the VAS was completed to improve understanding around the differences between the two groups. Scores on each scale were classified as a high score (≥8cm) or a low score (<8cm)\(^1\) and a chi-square (Fisher's Exact) test was conducted. These results can be found in Table 5.18. There continued to be a significant difference in terms of proxy ratings of Overall Health and in addition, there was a significant difference in the number of proxies scoring a high QOL in the Home PN versus the SBTx sample. There were more high QOL assessments by proxy in the SBTx sample. There was no significant difference between the assessment of high versus low Pain between the two groups.

Table 5.18 VAS Proxy Report (High vs. Low) Home PN versus Institutional SBTx

<table>
<thead>
<tr>
<th>VAS Scale – Proxy Report</th>
<th>Home PN Sample</th>
<th>SBTx Sample</th>
<th>ChiSquare (Fisher’s Exact) P value</th>
</tr>
</thead>
<tbody>
<tr>
<td>Quality of Life</td>
<td>n=15</td>
<td>n=6</td>
<td></td>
</tr>
<tr>
<td>High Score: n (%)</td>
<td>2 (15.4)</td>
<td>4 (66.7)</td>
<td><strong>0.0460</strong></td>
</tr>
<tr>
<td>Low Score: n (%)</td>
<td>11 (84.6)</td>
<td>2 (33.3)</td>
<td></td>
</tr>
<tr>
<td>Pain</td>
<td>n=15</td>
<td>n=6</td>
<td></td>
</tr>
<tr>
<td>High Score: n (%)</td>
<td>5 (33.3)</td>
<td>5 (83.3)</td>
<td>0.0635</td>
</tr>
<tr>
<td>Low Score: n (%)</td>
<td>10 (66.7)</td>
<td>1 (16.7)</td>
<td></td>
</tr>
<tr>
<td>Overall Health</td>
<td>n=15</td>
<td>n=6</td>
<td></td>
</tr>
<tr>
<td>High Score: n (%)</td>
<td>2 (14.3)</td>
<td>2 (33.3)</td>
<td><strong>0.0374</strong></td>
</tr>
<tr>
<td>Low Score: n (%)</td>
<td>12 (85.7)</td>
<td>4 (66.7)</td>
<td></td>
</tr>
</tbody>
</table>

\(^1\)Home PN sample size for Quality of Life assessment n=13
\(^b\)Home PN sample size for Overall Health assessment n=14
5.4.1.8 Self Ratings for Visual Analogue Scales

It was not possible to compare the VAS self assessment between the 2 groups as there was only one child in the SBTx sample able to complete the tool. The results for the VAS Self assessment can be found in Table 5.19. This child scored themselves 3cm lower on the QOL VAS compared to the mean score of the 4 children in the home PN group although the home PN scores had a wide range from 2.1 to 8.4 cm. The range of experienced Pain in the home PN group had a smaller range of 5.3 to 9.1 cm. The largest range was the perception of Overall Health and ranged in the 4 home PN children from 0.8 (worst overall health) to 9.7 (best overall health).

Table 5.19 VAS Self Report Home PN versus Institutional SBTx

<table>
<thead>
<tr>
<th>VAS Scale – Self Report</th>
<th>Home PN Sample Median (IQR) Range n=4</th>
<th>SBTx Sample Score n=1</th>
<th>Mean Difference</th>
</tr>
</thead>
<tbody>
<tr>
<td>Quality of Life</td>
<td>4.6 (2.4 – 7.8) 2.1-8.4</td>
<td>2.4</td>
<td>3.33</td>
</tr>
<tr>
<td>Pain</td>
<td>7.2 (6.6 – 7.5) 5.3-9.1</td>
<td>7.2</td>
<td>-0.07</td>
</tr>
<tr>
<td>Overall Health</td>
<td>7.0 (5.1 – 8.9) 0.8-9.7</td>
<td>7.0</td>
<td>-0.87</td>
</tr>
</tbody>
</table>

5.4.2 QUESTIONNAIRE ASSESSMENT ï MISSING AND REDUNDANT ANSWERS, CEILING AND FLOOR EFFECTS

All questionnaires were assessed in terms of the number of completed tools that were returned from both samples, the number of missing answers and ceiling and floor effects. Results for each of the tools analyzed can be found in Appendix N. Ceiling and floor effects were considered to be significant when greater than 25% of the respondents achieved the maximum or minimum score, respectively\(^59\). Both home PN and SBTx samples were included in the analysis.

5.4.2.1 ITQOL Proxy Report Questionnaire

The ITQOL was assessed in terms of 11 multi-item scales and 2 single-item scales. Twelve individuals completed the questionnaire except for the Physical Abilities scale which was only completed in total by 6 respondents. This was because of the option of ‘not doing yet’ for the developmental assessment, which was not considered a missing response. The Global Behaviour
and Getting Along With Others scales were completed by 9 respondents as it was only appropriate for children greater than 1 year of age. Of 12 respondents, there were 2 missing responses in the Growth and Development scale. Ceiling effects were >25% in one scale: Family Cohesion, a single item scale which had 33% (4/12) of respondents choosing the highest score. There were no significant floor effects present in this analysis.

5.4.2.2 CHQ-PF50 Proxy Report

The CHQ-PF50 was assessed in terms of 12 scales and 1 single-item scale. Nine individuals completed the questionnaire in total. There were 2 missing answers in the self esteem scale and 1 in the family cohesion scale, a single item scale. Ceiling effects were present in three scales with 3 of 9 respondents rating the highest score for Role/Social Limitations Owing to Physical Function, Owing to Emotions/Behaviour and Bodily Pain scale. Two of 9 respondents scored the maximum score in Family Cohesion and Parental Impact on Time and two of 9 patients scored the lowest minimum score in the Role/Social Limitations Owing to Emotions/Behaviour, however these scales were not considered to have significant ceiling or floor effects.

5.4.2.3 CHQ-CF87 Self Report

The CHQ-CF87 was assessed for 11 scales and 1 single-item scale. Four individuals in total from both samples completed the questionnaire. One item was missing from both the Self-Esteem and Behaviour scales and 3 answers were missing from the Family Activities scale. Three of 4 children did not complete the same item on the Family Activities scale due to a misprint on the original copy of the questionnaire that did not provide the response boxes for the item statement. This was noticed upon scoring of the tools and written in for the fourth respondent. Significant ceiling effects were present in 5 multi-item scales: Physical Functioning, Role/Social Limitations Owing to Physical, Emotional and Behavioural Function and Bodily Pain. All 4 respondents scored the maximum score for the Role/Social Limitations Owing to Behavioural Function. There were no floor effects.

5.4.2.4 PedsQL4.0™ Self Report

The PedsQL4.0™ Self Reports were assessed for all age groups in both samples according to the 4 scale, 23 item surveys. Nine children completed the scale with 1 redundant answer on the
Emotional Functioning scale and 1 on the School Functioning scale. This occurred on a Young Child survey (age 5-7 years). There were no ceiling or floor effects on any of the self report scales.

5.4.2.5 PedsQL4.0™ Proxy Report

The PedsQL4.0™ Proxy Reports were assessed for all age groups in both samples according to the 4 scale, 23 item surveys (the Toddler report has only 21 items). Sixteen respondents in total from both samples completed the surveys. There were 2 missing answers on separate surveys for the Physical Functioning scale. This was related to the item on the Young Child survey (age 5-7 years): “In the past one month, how much of a problem has your child had with taking a bath or shower by him or herself?” Both answers were left blank with a note saying that their child was too young to bath by themselves and that this was done by the parents. There were no ceiling or floor effects on any of the proxy report scales.

5.4.2.6 PedsQL™ Family Impact Module

The PedsQL™ Family Impact Module was administered in both samples with 21 respondents who completed the 36 item, 8 scale survey. There was 1 redundant response on the Social Functioning scale and no other missing answers. There were 5 proxy respondents who scored the maximum score (24%) on the 5-item Family Activities scale, demonstrating a high but non-significant ceiling effect. This scale asked proxies to assess over the past month as a result of the child’s health has the family had difficulties with communication, conflict, difficulty making decisions, solving problems or causing stress between family members. There were no floor effects on any scales.

5.4.2.7 Visual Analogue Scales - Self and Proxy Reports

The VAS were administered in both self and proxy completion for three constructs: QOL, Pain, and Overall Health. Six children were given the 3 self-report VAS for completion and 1 home PN child returned all 3 VAS blank without a reason provided. Twenty-one proxy respondents completed the VAS. One survey was returned with only 1 scale completed appropriately. The other 2 scales had more than one mark and were considered redundant. Another proxy failed to
complete 1 of the 3 scales. There were no ceiling or floor effects found on any of the VAS for both self and proxy respondents.

5.4.2.8 Functional Status IIR

The Functional Status IIR is a single scale with 14 items and was provided to all proxy respondents in both groups. Two incomplete questionnaires were returned with a total of 3 missing items. A ceiling effect was considered as an overall perfect score (i.e. 100) which occurred in 2 of 21 surveys but was not significant. There were no floor effects for this survey.

5.5 Primary Qualitative Analysis

5.5.1 FOCUS GROUP

A focus group was conducted with the parent proxies for the home PN patients. The focus group for the children was not possible as those old enough to participate lived too far from the hospital to attend. The home PN proxy focus group was conducted in a comfortable meeting room in the hospital with beverages and snacks available. The sessions were audio taped using two MP3 recorders which were then transcribed by a professional transcriptionist.

5.5.2 PARTICIPANT CHARACTERISTICS

The focus group consisted of five mothers and one father, who were parents of 5 children on home PN that were followed at the Hospital for Sick Children. The children represented by proxy were 8 months, 2½, 3, 4 and 8 years of age. Two of the 5 children had been on PN since birth. One child had been on home PN multiple times throughout life and the other two children started PN between 1 and 2 years of age and had been on PN for just over 1 year. At the time of the focus group, the children were receiving PN 5 to 7 days weekly over 10 to 18 hours per day. The focus group duration was approximately 90 minutes. One mother was unable to attend in person and participated via conference call. This mother had to leave the group discussion after 70 minutes. The focus group was lead by Julia Maxwell, social worker in the GIFT and SBTx programs and was referred to as the “interviewer” unless otherwise noted. Megan Carricato, co-investigator also assisted with note taking and probing questions. Pseudonyms were used for both the parent and child in order to protect their identity. Note that the parents refer to PN
(parenteral nutrition) in their discussion as TPN (total parenteral nutrition). For the purpose of these results, PN and TPN should be considered synonymous.

**Pseudonyms**
Parent #1 Jennifer – daughter Charlotte
Parent #2 Erin – daughter Maggie
Parent #3 Michelle – daughter Alisa
Parent #4 Jana – son Lucas
Parent #5 Kim – son Noah
Parent #6 Michael – son Noah

5.5.3 **DATA ANALYSIS AND FINDINGS**

To ensure accuracy of the transcribed document, the co-investigator listened to the audio recording and compared this audio data with the written content. Once accuracy was ensured, the manuscript was reviewed several times to become familiar with the content and to highlight any patterns or themes that were evident. These recurrent themes were documented. In order to obtain a more in-depth analysis of the rich data collected, thematic analysis was performed through a paragraph by paragraph review to search for repetitive or common statements made by families. These statements were then grouped or coded under broad categories and subcategories, keeping as much surrounding data to prevent the loss of context. The categories reflected basic issues faced by the families and related to the social processes of daily life that existed for these families. Relationships between and amongst the categories and themes were uncovered. Themes were described, revised, refined and at times discarded in order to generate the final themes with clear definitions and names with the aim to describe the proxy experience of having a child on home PN and their perception of QOL in home PN.

Following analysis of the focus group manuscript, themes were identified and separated into subcategories (See Figure 5.8 for Summary). These included discussion related to the pervasive impact of home PN on many aspects of life, the sense of medical responsibility in caring for a child on home PN, isolation and support, and the strive for normalcy in a very non-normal life. There was also discussion related to the children on home PN, communication issues and issues related to intestinal failure and home PN specifically. The final themes that were generated include gratitude and hope related to having a child with a chronic illness.
Figure 5.8 Qualitative Results of Home PN Proxy Focus Group
5.5.3.1 Quality of Life

The interviewer started the focus group discussion with a general question asking parents to describe what they believed the definition of QOL was for both their children and themselves. The parents responded focusing on different aspects of what they believed constituted QOL. A common focus was being the ‘best that it can be’ within the constraints of having a chronic illness and requiring PN therapy. Themes that arose included being happy, having the ability to be social, to have a normal life as well as the relativity and dynamic nature of QOL.

5.5.3.1.1 Happiness

Michelle discussed the fact that she felt overall, the most important thing was to be happy and how the happiness of her chronically ill child impacted the whole family.

Michelle: Basically the bottom line is being happy, I think. If Alisa’s happy, the family’s happy, we had a good day, all is good. <pause> Um, that summarizes happiness.

Erin also discussed the importance of being able to feel happy and introduced the topic of relativity. Life presents many individuals with various challenges and the feeling of happiness is contextualized within your own personal situation.

Erin: Yeah, I think being able to feel happy and having the best that we can have. Everybody’s circumstances are different.

5.5.3.1.2 Socializing

Another aspect of QOL that was felt to be important was the ability to socialize, including both interactions with the community at large and within the family. The value of social interactions was recognized however many of these children were restricted in their ability to be social because of the complex PN administration schedules and the risk of contracting an infection. Erin discussed the fact that Abby was limited in terms of her social interactions due to the risk associated with infection.

Erin: Socially is part of it, for sure. And the social interactions are different too. Abby hardly gets to be social because she gets so sick when she gets a virus or whatever.

After Noah was diagnosed with intestinal failure, he was unable to participate in family activities that included all of the children. Kim and Michael emphasized the importance of being able to interact in meaningful ways with their 3 other children for Noah’s QOL.
Kim: For Noah, in terms of his quality of life, Noah wasn’t always sick. And um I think to get him back to the point where he was able to do a lot of things, um where it doesn’t – like we have 3 other children – I mean we can do activities that involve all of our children.

5.5.3.1.3 Relativity

Given the circumstances that these families and their children had encountered over time, the concept of relativity was a very essential aspect of QOL.

Erin: So... you know, quality can be relative, relative to what you’ve experienced.

Michael also elaborated on this and how he and his wife have a completely different expectation about QOL after their experience with Noah.

Michael: So now we’re home, and you know, when we talk about standard of life, our standard of life has completely changed from what my sister and her family go through. So our standard of life is redefined.

The parents felt that these children have had to and will continue to endure many different challenges. Considering relativity allowed families to create expectations that were realistic for their current circumstances. Jennifer recognized that at times, in order to improve her daughter’s QOL, it may even mean a hospital admission.

Jennifer: So I define quality of life um in our home as what we can best do to make Charlotte’s life the best that it possibly can be. Um, anything we can do, so whether that be riding a bike, living at a hospital, all of those things quantify quality of life for us.

Erin and Jennifer both addressed the fact that the definition of QOL would be different for their chronically ill child in comparison to the siblings, other children and adults. Each of these children was considered to be unique in their circumstances which impacts QOL expectations.

Erin: Abby can’t have what her brothers can have in lots of cases, and do what her brothers can do... Trying to make – especially with a child that has chronic issues, the best it possibly can be.

Jennifer: I also think that it differs from child to child, ‘cause quality of life for my child who’s unwell might be different from quality of life for my husband.

5.5.3.1.4 Dynamic

Quality of life was also described in terms of its dynamic state which is defined as a usually continuous and productive activity or change\textsuperscript{164}. Having a child with a chronic illness can result in a significant amount of unknown and uncertainty on a daily basis. These parents agreed that the expectations or experience of QOL may change from day to day.
Jennifer: Well, I think quality of life differs from day to day, depending on what’s happening with um, especially with a chronically-ill child. [...] So um today for example, wasn’t quite as good as it was yesterday. And hopefully tomorrow will be better. So quality of life today for us was, thank goodness we’re not in the hospital, she got up in the morning, and all of her things that need to work at the moment are working. So <laugh> all of that is fantastic. Um, she didn’t go to school. She didn’t leave the house, she’s not well, she’s crabby. So you know... but tomorrow will be different. So in our home, I think that changes every single, minute to minute really, we don’t know what the next 5 minutes will bring.

5.5.3.1.5 “Normal”

The word normal is defined as conforming with, adhering to, or constituting a norm, standard, pattern, level, or type; typical. The term normal came up numerous times as having an impact on QOL. Many parents discussed the aspects that they attempt to change or modify in order to create a sense of normalcy in their atypical lives. Jana provided examples of activities that she engaged in with her son Lucas in order to create a sense of normalcy to improve QOL.

Jana: I think Lucas has a great quality of life [...] we do ‘Mommy and Baby’ groups, we have play dates, we try and go to most family events. And luckily everyone in our lives are pretty good about, you know, if anybody is sick or has been sick in the past couple of weeks they know to stay away from us. I think yeah, we try and do everything as normally as possible.

Interviewer: So is, do you think that the whole idea about quality of life, that part of, the important part of that definition is this idea of normal?

Jana: I totally think so. I think it’s, a lot of it is your perspective on what you think your child should be doing at any moment.

Michelle also mentioned trying to create as normal a life as possible within the context of having a child on home PN.

Michelle: Having as normal a life as you can possibly have, considering everything that’s going on. If hooking her up every night, oh that’s normal, that’s part of our routine.

Erin: That is normal.

Michelle: For us it’s just, okay, you do it, whatever, and you work around it. And so having that and going smoothly, that’s a good quality of life.

Kim brought up the concept of relativity again and related it to normalcy, emphasizing that normal is different for each individual and family.

Kim: When I think of quality of life, in terms of <pause> like I don’t want to say what is normal, cause what’s normal for different families, it just depends on your family dynamic. And we create our own sort-of normality, basically, in our household. This is what is normal to us now.
5.5.3.2 Pervasive Impact

It was evident through the focus group discussion that having a child on home PN has a pervasive impact on their lives, the child on home PN as well as their families. The schedule and clinical status of the child on home PN was the central focus of most decisions which impacted the families, the ability to have a normal marriage and also family cohesion. Due to the demanding nature of the therapy, the ability to be spontaneous and also to plan events was compromised. Additionally, their homes required many logistical changes to accommodate the supplies and schedule of this therapy which had a financial impact on these families.

5.5.3.2.1 Central Focus

The families all agreed that having a child on home PN with an unpredictable clinical status and a demanding schedule for therapy had resulted in the need to make decisions that centre on their chronically ill child.

Erin: So those are the kinds of things that we consider, every decision in our house basically, is how does it affect Abby. Like that’s what it comes around to.

Kim: It just means that you know our unit, our family structure, everything that we have to do is pretty much centred around if Noah’s able to do it.

Jana: I think it...I mean...our entire schedule revolves – exactly what the other moms are saying – totally revolves around what’s going on with Lucas, what his TPN schedule is like.

5.5.3.2.2 Family and Siblings

Having to centre all decisions around one child in an effort to provide the best care and safest environment for that child on home PN was described to have significant implications on the entire family. The parents discussed the feeling of isolating their other children in order to complete procedures related to central line care and PN infusion. The ability to book family vacations in terms of duration, location and frequency was also impacted. Activities that the other children in the family would be able to do were limited in order to participate in activities as a family. Jennifer discussed how over the many years that Charlotte has been sick, that this has had a profound impact on her other children.

Jennifer: And it has impacted them tremendously: psychologically, physically, in every way, shape, they’ve felt deserted, they’ve felt every possible unusual situation. They’ve felt short-changed some days.
Kim mentioned having to isolate the other children during procedures and that they, as a family, just try to cope with this situation as well as possible.

_**Kim:** But you know there are complications that come with it are, ...having to sometimes isolate the other children when we have to do his dressing care and so forth, those are challenges, but we’ve been trying to get through them as best as we can.

Erin discussed planning family vacations in the vicinity of the responsible medical team, although this was not unanimous within the group. Erin and her husband felt that their daughter was still medically precarious enough that the stress of travelling further was not worth it. This limited the types of experiences and vacations that she and her husband were able to provide the other children.

_**Erin:** We haven’t gone on a vacation, we haven’t gone...we have, we went to Niagara Falls when we had our appointment here. But they [the siblings] know vacation means somewhere in the vicinity of the GIFT Team. <laughing> That’s what it is, you know, the lion safari maybe, we’re going to the zoo.

All of the children had a central line for PN infusion which limited the activities that they were able to participate in. As a result, this impacted the entire family in an effort to avoid activities that would make the child on home PN feel left out or different.

_**Jennifer:** And it absolutely, 100% impacts the children, and my husband and I. Even a bike ride is complicated, right...we also have to remember that at the park there’s sand, and water, and all the things that [Charlotte is] going to have a fit about not being able to participate in. So you know, parks are kind of out of the question. And um you know water parks are absolutely not in the question.

_**Erin:** Beaches

_**Jennifer:** Swimming pools are not in the question. So it impacts the whole family. Going to camps, going to cottages, just aren’t things that are in our equation.

_**Erin:** Absolutely, I mean I agree with everything that Jennifer’s already said. And it does – my kids, we have a water park, like a 5 minute walk from our house, my kids went there, my boys went there once while she was napping last summer. She doesn’t nap anymore, so they won’t go. [...]They don’t even ask to go to the water park.

Lucas was Jana’s first child so for her and her husband, this was just what having a child was like because they didn’t know any different. For the other parents, this experience was vastly different than experiences with their healthy children. With their other children, they had participated in many activities that they chose to avoid to protect their sick child. Erin expressed that she believed her second child had been affected by this more than her older child because of the closeness in age to her daughter, Abby.

_**Erin:** [Middle child] hasn’t...I used to go to the library and do play groups. We can’t take that risk with Abby. So [middle child] therefore, has suffered more. [Older child] had done that and
As mentioned by Jennifer, the children had experienced a variety of emotions related to being the sibling of a sick child. These children had very different reactions expressed both internally and/or externally. Erin expressed feeling very lucky that her children had accepted their situation well and attributed it to not knowing a different kind of life.

**Erin:** And thankfully they’ve never complained, once. And I’m very proud of them for that. But they don’t know any different either. You know, this is what we do.

Erin also discussed how her older son has had to experience periods where Abby came close to not surviving and that he was simply grateful to have her alive.

**Erin:** Because I think he’s [older sibling] been through enough with Abby, he’s known that he’s almost lost his sister a couple of times, and he knows that. So he’s had to face things that most boys at his age don’t have to, and he’s a very serious, thinking child. He’s taking it all in. His brother’s younger, but also more, you know, go with the flow. [Older sibling], he hears everything.

### 5.5.3.2.3 Marriage

The marriages of these parent proxies were impacted as a result of having a child on home PN. Daily stressors, isolation of the family and themselves, having to be separated during long term hospitalizations, caring for the other children and having difficulty finding time to spend together as a couple were challenges described. Michael discussed the difficulty of being apart when Noah was first diagnosed and was in the hospital for an extended period.

**Michael:** When Noah was in the hospital, Kim was at the hospital with him, so I was at home with the other children. So all of that on me was a lot, as well as the mental stress of just being there and dealing with Noah. Not to mention, we’re split apart. Um, so that was...that was very hard.

Kim and Michael described a traumatic event that they experienced which resulted in moving Noah into their bedroom.

**Michael:** He’s in our...he’s in our room. We tried having him in the room beside us. And one night, we didn’t get to him in time. By the time we got up, he completely climbed off the bed. **Kim:** (Overlap) He had a barrier. He had a barrier...he had a monitor. And he was playing in his blood. His line, he had pulled it out, and he was playing in his blood. **Michael:** So we had to rearrange our whole bedroom. **Kim:** Our whole house really. **Michael:** So now his bed is right beside ours.
In fact, each of the parents with younger children stated that their children slept in their bedrooms in fear of their child pulling out their central line.

**Jana:** Agree. Lucas also sleeps beside us in our room for the same issue about the line.

Erin discussed how she and her husband put Abby into their room after coming home with a central line. Prior to the initiation of home PN she was sleeping in her own room. However, she put this into perspective by comparing it to their prior situation when Abby was in the hospital and she and her husband were separated.

**Erin:** Abby does too. She’s in her crib still because she’s tiny, but she’s going to get to that point. But I couldn’t sleep if she wasn’t, cause exactly, I know, when she’s old enough she could be in a bed. But I know she wouldn’t wait for us to get there, if we happened to not hear, cause she’d just be coming to get us and climb down. So she’s actually still in her crib, beside our bed. But people are like, ‘She’s almost 3, and she’s in your room?’ She wasn’t when she wasn’t on TPN, but she is, so, that’s life. Pretty minor compared to my husband sleeping at the hospital for 6 months. Me spending every day at the hospital for 6 months and not ever being together.

### 5.5.3.2.4 Spontaneity and Planning

The parents also discussed another aspect that impacted family life and the other children with respect to the inability of being able to engage in spontaneous outings or even planned outings. The children were on scheduled infusions of home PN and although there was a certain amount of flexibility, durations of 10-18 hours result in limited amounts of time to ensure the therapy is provided. When events are planned the parents need to try to work the day around the PN.

**Jennifer:** Having a child on home TPN impacts our family tremendously. So it’s... everything has to be timed, and there’s no real deviating from that time [...] because everything has to, nothing can be really spontaneous... Um, planning every... planning our whole day around the time off that she has, can be complicated, because if we’re at the zoo, or wherever – a birthday party – it all has to be, it all has to coincide with what works best for Charlotte.

Jana expressed also that their days were determined by Lucas and that a change in clinical status would change their plans.

**Jana:** We are led by whatever Lucas is doing at the moment. If Lucas is sick, well that might change our plans.

Taking vacations was a very important aspect for these families in order to spend quality time as a family. The parents had differing comfort levels about taking vacation. Erin previously mentioned that they were uncomfortable travelling too far from the medical team that cared for Abby. For those families who had tried to take a vacation, they described the challenges
involved such as having a refrigerator for storing PN supplies, having the capability to run the technology and also the arrangement of events around the infusion schedule. Michelle discussed their single experience with an overnight family vacation.

Michelle: And vacation, like, you took a vacation! [to Jennifer] How did you do that? There’s no way. People always, I think they don’t understand, like ‘Why don’t you go on vacation?’ ‘Do you know the logistics involved?!’ Carrying the TPN, the supplies...we did one night in Niagara Falls, and we’re never doing that again. It is so not worth it.

Jennifer had more experience with taking vacations and suggested that it was more worthwhile to go for an extended period due to the hassle and amount of planning involved. However, she also discussed other aspects of vacation planning that made it complicated to travel with a child on home PN.

Jennifer: We have to make sure there’s a sterile environment, which going on vacation was a bit challenging, because we always had to call ahead to make sure that there was a nursing station or something like a nursing station nearby.

5.5.3.2.5 Family Cohesion

Family Cohesion was a very important aspect in this focus group. There was unanimity in the fact that despite all of the challenges, each family vocalized that they had chosen to take all of this on as a family unit.

Jennifer: We’ve decided to tackle this as a family. That was a decision that my husband and I made.

Erin: [...] because we too tackle this whole thing. We’re a family group. We don’t even come here for our appointments without bringing the [other children], ever. They’re...we’re in this together. And they know it.

Family cohesiveness was expressed by these parents in different ways such as the entire family avoiding activities that the child on home PN was unable to partake in, having the family help with the care of the child and also expressing the importance of time together as a family.

Michael and Kim described how their family has worked as a team.

Kim: And we have 2 older children...and they’re excellent. Like, they’ve learned to even help us change poop. [...]And if by chance you know we’re – cause we both work – if we’re headed out to work, and there’s a period of time where ...my oldest son, he’s with Noah for about ½ an hour before we come home, cause I don’t work far from home. And let’s say there is an accident, he will just take responsibility right away and help out.

Erin also described what she felt was the positive impact that these experiences had on one of Abby’s siblings.
Erin: [...] he makes little comments about just, when we’re together as a family, that he’s really happy about that. I don’t think that most almost 8 year old boys recognize the value of family as he does.

5.5.3.2.6 House and Home

The parents discussed how bringing a child home on PN required a significant amount of adjustment to their homes whether it be related to the layout, the storage and refrigeration or even the electrical work. Jennifer and Michael discussed the need to reorganize their home to accommodate the technology and to ensure the safety of their child for monitoring purposes.

Jennifer: Um, and just... just the supplies. We had to, when Charlotte first came home, she had 5 TPN machines, or 5 different machines. So we had to have her whole electrical in her room rewired, because it was not set up to equip that many, that much machinery and electricity. Storing all of her stuff takes up so much room. And we’re in such a small house.

Michael: So we had to rearrange our whole bedroom.
Kim: Our whole house really.
Michael: So now his bed is right beside ours.

In addition to the restrictions imposed by the home PN schedule, the families discussed the need to accommodate deliveries of PN solutions and supplies from the home PN companies.

Michelle: But the deliveries, like making sure you’re home at certain times to get the deliveries of the TPN. We’ve got a whole fridge now, just for TPN. Things like that. You just adapt.

The families discussed the strategy of organization of ordering and delivery to cope with this additional demand. This allowed the families to regain some control in scheduling their lives.

Kim: [...] everything is um pretty much organized. So 9 o’clock we get the call for our supplies. The supplies come at around 3, 4 o’clock. We do his dressing changes once a week, on Monday.

5.5.3.2.7 Finances

Despite being very grateful for the fact that the Ontario Health Insurance Program (OHIP) covered the PN and the supplies required, the parents described the financial implications of having a child on home PN. Non-therapeutic costs incurred included the cost of parking at the hospital for multiple appointments and hospital stays, replacing bedding and mattresses due to bedwetting and leaking diapers, home modifications to accommodate the therapy, running laundry daily multiple times daily etc. Some of the families had chosen to have one parent stay at home to care for the child, especially for the younger children. Having a single income also
presented financial challenges. Jennifer summarized the financial challenges very well and the other participants nodded enthusiastically in agreement.

Jennifer: ...not that it costs us money to have them on TPN, but to have Charlotte’s room rewired electrically was extremely expensive. And to have bedding and to replace mattresses every year, and to have um respite care and all of that kind of stuff coming through the house...the cost of driving into Toronto, and the cost of parking...and although we get parent rates, and there are other things that we can do, from time to time, we’ve been doing it for 8 years. So you know and also, when you are doing all of this, it’s difficult to work, and go to all of these appointments. Um, so holding a job is extremely complex. And then financially living without that second income is even more complex. ...I think that the home TPN is fantastic, but that there’s a million other financial things that, and even you know, laundry soap and the cost of running a dryer all the time. Those are all tiny little things that add up and add up and add up every day.

5.5.3.3 Medical Responsibility

The role of a parent was described as being significantly different from parents whose children were on home PN. The maternal and paternal roles had to be incorporated with a nursing role. This new role encompassed a significant amount of medical responsibility, required diligent care to avoid serious complications, to learn to differentiate everyday symptoms with serious symptoms requiring medical attention, to learn to anticipate problems and to monitor their children for serious side effects of PN therapy.

5.5.3.3.1 Diligence

Provision of PN via central catheter requires extreme diligence in sterile technique to prevent mechanical and infection related complications. This is the most essential aspect of the home PN teaching that these families are given in preparation of taking their child home on PN and is regarded very seriously by these parents.

Erin: And [my husband] and I are the same, he actually does the hands-on part, almost all of it, cause he’s...I’m ok now, but I wasn’t comfortable at first. I did the training, I know...I’m the sterile police in our house. He’s doing the hands-on, and I’m like, ‘You touched that!’

The families felt that they were best educated and prepared to perform this task and avoided help from other sources due to a lack of trust, even with home nursing. The repercussion of breaking sterility was significant and parents expressed putting pressure on themselves to keep their children out of the hospital.
Michelle: We haven’t been in the hospital – knock on wood – in a while, and we want to keep it that way. We’re very diligent.

5.5.3.3.2 Assessment of Symptoms

Like all children, a child on home PN develops many typical pediatric infections, however the parents expressed very high stress levels when it came to common symptoms. This was due to the risks associated with having a central line and the fact that a simple fever can also mean a serious central line infection requiring intravenous antibiotic therapy or removal of the central line. The parents expressed feeling the pressure of having to differentiate typical versus serious symptoms and also the significant medical work-up that was required for their child in the presence of these symptoms. Jana illustrated this point with a recent experience.

Jana: Um, a one-year old gets a fever for teething, cause they’re teething, or a runny nose. And maybe for us, a typical child that’s not a big deal, give them some Tylenol or Tempra and kind of get on with your life. But for example, Lucas had an ear infection last week, and went to the hospital, went to the ER, spent the whole night in the ER until they could figure out what was going on, had blood work done from his PICC line, had blood work done from his hand. Where if he was a typical child I probably would have taken him maybe to the paediatrician or the walk-in, got him on antibiotics, it would have been taken care of you know right away. You aren’t worrying the whole time that something else is seriously going on.

Erin also described a recent experience where she had issues with flushing her daughter’s line.

As a result of past experiences, Erin was anxious that something very serious was happening.

Erin: And her CVL flushed in the morning perfectly, and at night wouldn’t budge, wasn’t doing anything. Um and you know you’re 5 hours away. And it’s late, because we actually were, she wasn’t supposed to be on that night, and we decided to, we usually flush in the morning. But because she was going to bed late, decided, let’s flush her tonight in case she sleeps in (yeah right, but in case). And then you discover, oh great, here it is. It’s 9:30. It’s not moving. We’ve already experienced the panic of a blood clot. Is it completely clotted? But it worked this morning. So you do a cap change on your over-tired baby at 10 o’clock at night.

5.5.3.3.3 Monitoring for complications

Long term PN has numerous complications that must be monitored for including infectious, thrombotic, metabolic, and hepatic effects. Although these children were monitored closely by the medical team as outpatients, the parents recognized that they were the first line of defense. They expressed awareness that they carry the responsibility of anticipating and detecting these problems.

Jana: You have to be on your toes all the time.
Michael: Exactly.
The families placed very high value in being at home with their child and family and tried to avoid hospital admissions as much as possible. Because of this, they all agreed that they play a ‘time game’ in their minds which involved monitoring their child when they were unwell and hoping and waiting to see if they improved in order to avoid both an admission and a visit to the emergency room. Jana and Erin both described the ‘time game’

**Jana:** [...] you play the game in your mind: if we wait a couple of hours to go to the ER, is this going to be life-threatening?

**Erin:** And you know, now it’s ‘yeah, we’re not at the hospital’. Everything’s based on when she’s sick. We’re not where we need to go to the hospital, yet. We’ll just call and check in, but we’re not in the hospital. But you know, and that’s huge. But you’re always living like that in the back of your mind that, kind of almost always only one step from there! <laugh> Like all it’s going to take is a gastro, or some little bacteria that decides to be where it isn’t supposed to be, or a CVL that decides to be blocked, or a blood clot, or a...you know, many, many things. And everyone’s probably totally unique in their situation in some ways, but we’re always sort of one step away.

In the above quote by Erin, she expressed the feeling that they were always one step away from a hospital admission. This exemplified the uncertainty and stress that was associated with having children with a tenuous clinical state and being responsible for detection of these changes. Erin also mentioned the stress associated with having to monitor for side effects post-procedure.

**Erin:** And even, one of our questions has been, the other times when her CVL has been done, she has been in hospital for the initial healing of it. Now we’re just going to have hopefully a 4 hour recovery, and then we’re on our own. And it’s just been put in and so it’ll be more fragile. And how much bleeding is too much? You know, all those things that we have to deal with on our own [...].

### 5.5.3.4 Isolation and Support

To be isolated is to be set apart from others; place alone and was a theme that emerged upon the iterative analysis of the focus group transcript. Isolation was present for many different reasons and was discussed by the parents in context of having to isolate their child, themselves, their families and also related to physical distance from the medical team. The parents also described self-imposed isolation due to various concerns about their child’s wellbeing and as the resistance to use respite due to a lack of trust. In order to prevent isolation, they discussed the importance of support for and from their own families, friends and the medical team. The families were very
interested in improving support and communication for other families in the future in order to assist with a smoother transition to home PN and to minimize feelings of isolation.

5.5.3.4.1 Child on Home Parenteral Nutrition

Due to the significant impact that developing an infection can have on the child’s intestinal tract that is on home PN, there was a common theme of trying to protect their child from others who were unwell. All parents stated the fact that their family and friends were aware that they were unable to visit if they were sick.

Jana: [...] I mean we do probably shelter him a little bit more from germs, and people who are sick know they are not welcome anywhere near our house.

Kim: Um, I mean he is very sheltered as well because of his illness...They all know that if they’re sick – and there’s a lot of children, there’s 9 or 10 nieces and nephews and 2 on the way – but if anybody’s sick, as they often are, they know not to come over.

Erin talked about the fact that it was challenging to find a balance in life to provide quality while avoiding contact with others who were sick.

Erin: ...I used to go to the library and do play groups. We can’t take that risk with Abby...I’m scared to death to go to the library story time ‘cause everyone’s coughing up a storm. You know, you’re just eww. And I know this is going to mean 2 months of being sick with Abby in hospital, it’s not worth it. We have lots of books at home. [...] So we over-protect, or I don’t know if it’s over-protect, but we have to protect her. But, when she gets the chance, she’s just miss social butterfly. But it’s always – I think I often say we’ve got to weigh the balance: pros, cons for Abby, risk of getting sick versus...having a good life, you know.

Another element of isolation was related to the fact that these children with intestinal failure on home PN are a small demographic in the world of chronically ill children. Other larger disease groups, for example cardiac disease, renal disease or cancer, often have a larger population and as a result more resources available. This allows the organization of summer camps and events directed to the disease population. For the home PN group, there was less opportunity for these children to be engaged with other children who had similar experiences.

Jennifer: And that’s the other thing is that I think it’s a bit isolating for Charlotte, because she can’t go to camp (unclear), she can’t go to transplant camp, there’s no GIFT [intestinal failure/home PN] camp. She doesn’t really fit, she’s not ticked off in any little box anywhere.

5.5.3.4.2 Family

As previously described, there was a pervasive impact on the family. Isolation was a factor described in terms of the siblings and the entire family. During long term hospitalizations, the
siblings were often left with only one parent or extended family. On a more regular basis, parents described the challenge of having to isolate the other children during sterile procedures.

**Michelle:** And you know if we have to hook up Alisa, [younger sibling] has to sit and play alone, while we’re doing a sterile procedure, whatever. Hopefully he won’t get into trouble while we’re locked in the room.

**Kim:** We do his dressing changes once a week, on Monday. The children know, the other children know that they have to sit down, read a book, watch TV, for you know, at least about a half an hour. But you know there are complications that come with it are, you know a lot of laundry, having to sometimes isolate the other children when we have to do his dressing care and so forth, those are challenges...

Jennifer worried about the impact that having a chronically ill child with high needs would have on her other children. Despite an extensive effort, she found limited and expensive resources for her other children.

**Jennifer:** And so there are support groups out there. They’re extremely difficult to find. And a lot of them are, um the ones that my kids have, we’ve sought out, and we’ve been able to, they’re expensive, first of all. And um because we only get the information from [other healthcare centre], a lot of them deal with different issues than what my kids have to deal with. So we are in a family-centred care environment, and I would just like to see a little bit of support for the children, and even the fathers and mothers, or whoever is going through this, that needs a little bit of support, a little bit of understanding, or perhaps being with other children that are going through this...Just to be able to be with other people who really understand it. Rather than, even finding help for them, one-to-one, was extremely complicated. It was available for Charlotte, like everything else is. But it was not available for the other children.

Some of the parents described the difference of self-imposed isolation versus having family nearby for a sense of love and support.

**Kim:** I think we try um and still enjoy ourselves, because we, for the first little while when he was sick, we went nowhere, we had no-one over. But things have changed, and our family is very intertwined, including our extended family.

For others, extended family lived out of province which reduced available support and also made it challenging to stay connected. Erin discussed the impact of having their extended family living out of province.

**Erin:** It’s even affected my extended family in that they know if they want to see us, they have to come to us, where we used to always go to them, at least twice a year. So, thankfully my parents are super that way. You know, I’ve seen my brothers once in the last couple of years, just because of their logistics you know, too. So it affects my... our extended family.

Most parents expressed satisfaction with the emotional support they received from their family and friends. However, Michelle had a different experience and mentioned that she and her husband felt unsupported and isolated from family and friends.
Michelle: A lot of friends and family, maybe cause they don’t understand, they’re uncomfortable with the situation, they kind of alienated themselves from us, if you want to use that word. It hurts. But we can’t change their attitude, so...[...]

5.5.3.4.3 Caregivers

The parents who were primary caregivers of these children expressed minimal time for social activities due to the demands of caring for their children. Some believed that it was self-imposed due to the lack of trust of other caregivers, including other health care professionals. By not accessing respite care, the families were unable to leave the home each night while the child was on home PN.

Michelle: We haven’t been in the hospital – knock on wood – in a while, and we want to keep it that way. We’re very diligent. We don’t get nurses, we do everything ourselves. We don’t go out. We only have my mom really to babysit. We don’t trust anyone to take care of her, except us. So I don’t know if we impose that on ourselves, where we can get more help, but we choose not to. So we do isolate ourselves, I guess.

Jana: And I do 100% agree with the isolation comment that one of the moms made. [My husband] and I definitely both choose, I think a lot of the time, to isolate ourselves. We do not have anyone, no-one’s ever watched Lucas for us. One of us will go out at a time, and the other one will stay with him. But usually we do things as a family, the 3 of us.

The parent who was the primary caregiver of the child on home PN was thought to perhaps have the most isolating of jobs. Some of the families were able to support their child and have both parents back at work. However, for those caregivers that stayed at home to care for their child, there was a perceived loss of identity.

Erin: [...] it’s isolating for us all, in a way, maybe especially for the parent that stays home, I think. Like I sometimes say to my husband, ‘You know what, you go to work and people recognize you as a person.’ And I’m at home.

Being the primary caregiver appeared to promote a constant focus on the child and was described as being very stressful. The caregiver who was isolated with the child had to deal with the potential complications and side effects. When something happened, they described a lack of support due to having only themselves and their spouses to discuss issues with. This sometimes led them to wonder if their reactions were valid or if they were simply too focused on an issue.

Erin: Because that’s the negative part – it’s the isolation, the loneliness, that sometimes when I, ‘Am I crazy? For being this stressed.’ Like, maybe I’m taking it too much. But you’re really not. Like, it’s normal...
The use of respite care is intended to alleviate some stress for parents and to allow them to have some time to themselves. Many of the families expressed a lack of trust in others, both family members and healthcare professionals and were not willing to use respite care.

**Jana:** If Lucas needs to get home because he’s on TPN, then we’re at home because he’s on TPN. If he’s off TPN, then we’ll go out for day trips, but no, we...there’s no way we would leave him with anybody else.

Kim and Michael would not allow others to do the PN care, however on rare occasions they allowed the family to watch Noah so they could have time together as a couple.

**Kim:** But things have changed, and our family is very intertwined, including our extended family. A lot of them want to be able to help, and they ask me, ‘What can I do? Can you teach me how to do it?’ Although we don’t trust anybody else to do it, the fact that they’ve asked <laughing> and are willing to watch Noah, we hook him up, everything is um pretty much organized.

Erin explained that although they were rarely willing to use respite as a support, it had been a positive experience when they had done it.

**Erin:** It does feel good when you do, though. We have done it a couple of times and it does feel good.

After many years of not utilizing respite services and having difficulty keeping up with the responsibilities in life with minimal support, Jennifer finally decided to trial respite. After extensive teaching and the assurance that the nursing care was competent, she felt that she had gained some control back in her life with that additional support.

**Jennifer:** At our home we now have a nurse, full-time, 6 days a week. And after many, many, many, many days of sitting with her and being sure that she does everything the SickKids way, I have finally allowed her to take over Charlotte’s, most of Charlotte’s care. So um my life has changed dramatically over the past year. Um, I know that the nurses are diligent. I know that they are taking care of her. They have Charlotte’s best interest at heart. And we only have 2 nurses. So, that being said, she’s been septic twice since November. But anything, like I can’t say that it would be any different if I were doing it. We also have a PSW [personal support worker] who comes in. And she just gives Charlotte a bath, and cleans her room, and changes her bed, so at least we get one day of not having to do it. And she folds her laundry. [...] And I like Charlotte’s room immaculate, free of dust, free of any sort of other thing that could possibly be brought into that room. And the PSW just gives me a one day break to be able to do that. The nurses don’t do any of that kind of work, or change her bed or any of that kind of stuff. But they can do line care, and order the supplies, which I was just finding all of those things were, and getting up with Charlotte every night, and dealing with the 2 other children, and my husband works [out of town], we have no family support here, um was just too, very difficult. And I was working full-time at the time. So we needed, I needed something. And it has worked out so well. So that has helped out a lot.
5.5.3.4.4 Physical Distance

Isolation was also both imposed and experienced as a result of physical distance from the medical team. The family that did not live locally expressed discomfort about the distance to the primary medical team and articulated a fear of something happening so far away.

*Erin:* On the other hand, I’ve experienced huge amounts of stress, as we all have, when I’ve had those things going wrong. We’re a 5 hour drive away. ... And her CVL flushed in the morning perfectly, and at night wouldn’t budge, wasn’t doing anything. Um and you know you’re 5 hours away.

This also transferred to being afraid to vacation and travel to visit family because of the distance from the familiar medical team. The parents expressed significant frustration and negative experiences with other medical teams they believed did not understand their child. For some families, having a child with intestinal failure which is managed in limited centres has resulted in a lack of confidence in other hospitals and stress that made trips feel not worthwhile.

*Erin:* And so you know, we have all those, the whole family knows we can’t. I’m from the Maritimes, we haven’t been home for 2 years. And I don’t even...I know it can be done. But for me, the issue of the possibility of one of these things that we all live with, the possibilities of happening, happening when I’m that far from the people that understand what, well nobody really understands her, but understands that we can’t understand Abby. I just... I can’t face that stress.

5.5.3.4.5 Supporting Other Families

The parents expressed the value of having the focus group to voice concerns with individuals who appreciated their experiences. It helped with reducing the sense of isolation as well as acting as a support. It was also beneficial for staff as well to understand their point of view and to truly appreciate their daily challenges. Jana and Michael both emphasized that their experience of participating in the focus group was a positive one.

*Jana:* No, you know what – this actually feels really, really good. This is the first time I’ve ever been able to speak to other parents. So, it feels really good. It would be nice to be able to do this in some other way.

*Michael:* I think this, um I don’t know if this really answers your question, but I think this forum is going to help the next group <lots of agreement from parents>. Because even me just being here, we’ve never sat with anyone that has a child with a disability. So it helps, whether it’s the exact same [...]
Erin: And I’ve often thought that one thing that would be nice – this is maybe an aside – but as um...a support system, if there was some sort of even, blog situation, internet, news group, whatever you want to call it...

Jana: I was just gonna say that!

Erin: ... that we could all be part of. But um you know, even something like that, so that Jennifer could check in and go, ‘Oh, you know what, I feel your pain.’

Although family and friends made significant attempts to understand their experiences, the parents felt that it was not the same as receiving support from others who had children on home PN.

Erin: It’s more just, I think, the emotional support, not ‘what am I going to do?’

Jennifer: Even to know that you’re not cracking up.

Erin: (Unclear) I’m not cracking. Because sometimes you’re so stressed by it, cause you really are worried and you actually do have a reason to be that worried. But because you have no-one else other than your spouse that you can talk to. Like none of my friends get it, really. I can’t call them up and say, ‘you know what, I’m freaking out because her line doesn’t flush!’ They’re like ‘What? That’s terrible.’ They mean it, like that’s terrible, like wow, that must be stressful. But they don’t get it.[ ...] I’m not going to take medical advice from another parent. But you know, some of the last couple weeks even, being able to say something like, you know what, it is a panic when the line doesn’t work. And I get what you’re saying. And you are feeling stressed, but you have a reason to feel stressed. So it’s okay. It’s okay to vent to me because I can say ‘Yeah, I got you.’ It’s that kind of support.

Similar to the theme above, the parents also felt supported by the medical team but believed that other parents would provide more valuable support.

Jennifer: [...] I really, my...my interest is to help those behind us as well. And perhaps families that English isn’t their first language, and are going through this extremely difficult time.

Michael] the next parent that comes along, at least something can be given to them to say, ‘You know what, this is what you can expect. This is how you’re going to have to adjust your house. This is how it’s going to change your finances, structure within your home, vacations...’ anything like that. At least it’s not a shock or you don’t know what to expect. [...] I don’t want to have to try and feel my way out and learn everything new.

Jennifer: This is complex stuff.

Michelle: It’s different coming from a doctor or a nurse, versus someone who’s going through it, day to day.

5.5.3.5 Strive for Normalcy

The desire to have a normal life and the perception of the impact that normality has on QOL emerged in many facets of this focus group, starting with defining the term QOL. Not only was this described as a family-driven goal but many medical therapies and treatment plans aimed at providing the most normal life possible for these children. In an effort to obtain a normal life,
the parents discussed various ways to try to achieve this goal in terms of their marriage, family and for the children themselves.

5.5.3.5.1 Adaptation

Achieving normalcy in a life with an intensive treatment regimen required adaptation in planning and living. The parents described the goal of achieving a life as ‘normal’ as possible within the context of their own experiences and reality. Vacations were a topic discussed at length during the focus group as they were viewed by this group as an important event for the family to enjoy together but also something that required a vast amount of planning. 

Jennifer: We have to make sure there’s a sterile environment, which going on vacation was a bit challenging, because we always had to call ahead to make sure that there was a nursing station or something like a nursing station nearby. As she gets older, we tend to be a little bit more risky, but knowing that it could cause us some definite problems if we’re not cautious. Um, how we carry the TPN, how we hook it up, where we do it.

Kim: If we are planning a vacation, it will be difficult, but <long pause> … but <laughing> but we’ll make it happen. And so that means it requires more planning, a shorter vacation, still, it’s something that we can enjoy as a family. And we’ll do that, we’ll make it happen.

5.5.3.5.2 Family

As previously outlined, the child on home PN was often the central focus in decision making. For these parents, there was a struggle to balance the well-being of their sick child and also ensuring that all of their children’s lives were as normal as possible. There was recognition that the siblings miss out on many things but the parents try to make up for it in other ways.

Michelle: I’m pregnant. I have another little guy. So we haven’t touched upon the issues – and I’m kind of worried in the future, how that will affect the boys – he’s a boy too <pointing to pregnant belly>. So we try to make their lives normal too.

Erin: So we don’t go and even, the odd time we have actually made an exception, when the boys got invited to a hockey game with a friend. And it was (unclear), you know, the odd time. But usually they know we don’t do that in the evenings. But we try to make up for it in other ways.

Kim and Michael have found the support from their family and the family’s very close involvement extremely helpful to have a sense of normalcy in their lives.

Kim: And having our family not really isolate us because of the situation also is huge for us, because we’ll allow them to come over...And um I think to get him back to the point where he was able to do a lot of things, um where it doesn’t – like we have 3 other children – I mean we can do activities that involve all of our children.
Jana and her husband did not have other children when they had Lucas, so they did not have a frame of reference for normality with a baby. As a result, Jana expressed that this has been their normal.

*Jana:* But I think maybe it’s a bit different for [my husband] and I because Lucas is our only child right now, so he’s our first child. We have never had any other experience. I had Lucas, he was in Sick Kids for the first several months of his life. And now we’re at home, dealing with all these issues at home. So I think for us, it’s kind of just, this is, for us, what having a child is like.

### 5.5.3.5.3 Marriage

Attempting to maintain some sense of normalcy in their marriages was important amongst these families. The care regimen for families of children on home PN, as previously mentioned, involved a certain level of isolation. These parents have expressed discomfort in having other people take care of their children due to a lack of trust. Additionally, most of the children have been sleeping in their parent’s bedroom which makes it difficult to have a normal relationship. Some of the parents have just accepted that this is what it was like for now. Kim and Michael talked about their commitment in having even just one night together, every so often, to focus on their relationship.

*Kim:* So it’s huge, just having more people involved in Noah’s daily TPN care, and even just his care, in general, that allows for us to, you know, go out on dates sometimes. I mean not often, maybe once every two months or something like that. But still.

### 5.5.3.5.4 Child on Home PN

The children themselves had been dealing with chronic illnesses requiring treatment that was very invasive in their daily lives. Having a central line with a PN infusion prevents a complete sense of normalcy. However, many of these children grow up with this therapy and adapt quite well due to a lack of other experiences or frame of reference. The parents discussed the fact that they try to engage in normal activities to allow their ill children to experience life as other children do.

*Jana:* ...But, we do ‘Mommy and Baby’ groups, we have play-dates, we try and go to most family events. And luckily everyone in our lives are pretty good about, you know, if anybody is sick or has been sick in the past couple of weeks they know to stay away from us. I think yeah, we try and do everything as normally as possible [...]. We are led by whatever Lucas is doing at the moment. If Lucas is sick, well that might change our plans. If Lucas is feeling great then we go to the zoo, or we do, you know, whatever other kids his age would be doing.
Kim also discussed the fact that striking a balance was essential so that Noah would not feel different from all of the other children, including one of his siblings who was very close in age to him.

**Kim:** It’s finding a balance, and making him feel that he’s not different from the other children. Um in terms of school for example, we’ll put him into a school with his sister, they’re going to be starting at the same time, they’re 10 months apart.

Jennifer also spoke about her gratitude regarding the medical team that cares for her daughter and their focus on incorporating normality in her life.

**Jennifer:** [...] when we got taken on by the GIFT team, the whole focus was how do we get her out of the hospital, how do we get her at home. How do we get her to be able to rollerblade and do all these other things? And we’ve been on, I don’t even know how many vacations so far. We just pack it up and head on out!

### 5.5.3.6 The Children on Home Parenteral Nutrition

The children on home PN, as many other children with chronic illnesses, are often confronted with serious challenges at a very young age. These parents acknowledged that these children show immense strength and adaptation in the face of adversity.

**Michelle:** I think all these kids are just so amazing. Like, it’s their positiveness. They’ve gone through hell. They’re always so...I don’t know what the word is. So upbeat, so gung ho, they enjoy their life, as they should. And I admire all of them. That’s why we fight so hard for them, I think, ’cause they’ve gone through so much.  
**Erin:** Cause they’re fighting too.  
**Michelle:** Yeah Exactly. So, we fight with them.

There were many aspects of their lives that are impacted by having to be on home PN. Unfortunately a sample of children who were age-appropriate could not participate in a focus group to get a direct view of what life was like for the child on home PN. The parents expressed their opinions on the impact of PN on their child, physically, socially and emotionally. In terms of going to school and developing friendships, only one child was of school age and had experienced these challenges. Thus, the analysis was based on the opinion of one parent.

### 5.5.3.6.1 Physical Impact

Jennifer felt that the reinitiation of PN for her daughter, Charlotte, had such a positive impact on her from a physical standpoint. Her energy level was significantly improved which allowed her to participate in many more activities and thereby improve her QOL and also that of her family.
Jennifer: There are a lot of things, a lot of different ways that home TPN impacts the family. But at the same time, she’s so much better on home TPN than she has been. So the idea of being able to live out of the hospital and that she is a bit stronger, that impacts our family in a different way, in a positive way. Where she has a little bit more energy to go for that bike ride, that, you know [...] 

However, due to intravenous fluid running through the night, a negative side effect of bedwetting was said to be a significant problem for these children. The parents expressed the need to change bedding often and to do multiple loads of laundry daily due to enuresis.

Jennifer: There’s also the issue of um bed-wetting. And so we spend half of our day doing laundry, because Charlotte is soaking through all of her sheets and everybody else’s.

Kim: So if he wakes us, and I change the diapers, sometimes I don’t get to him, and they’re soaked, the beds are soaked.

5.5.3.6.2 Dietary Restrictions

In order to avoid excessive intestinal output, many of the children were prescribed hydrolyzed formulas or restricted diets eliminating simple sugars and fruit juices. Michael discussed the challenge of having to explain to his young son that he was unable to have certain foods and beverages.

Michael: Just in line with that, um within our family, there’s a lot of nieces and nephews or cousins. You have a birthday party, and the birthday cake comes out. Noah can’t eat that. So then he sees everyone eating it, or sees everyone drinking you know fruit juice or this or that. And to explain to him, ‘No, you can’t have that.’ You know, he doesn’t care what the reasoning is, he wants it. And to always have to, um even at the dinner table, our drinks are in the kitchen because if he sees a table full of drinks where he only gets this water bottle, you know for the day. That’s another thing that adjusts everything.

5.5.3.6.3 Activities

Although there were restrictions on what children were able to do with a central line, in order to create a sense of normalcy, the medical team and parents have encouraged the children to participate in as many things as possible. Jennifer mentioned how rewarding it was to see her daughter participate in many activities.

Jennifer: And I think that the GIFT team, I know in our situation, have waived and put aside and allowed Charlotte to do the many crazy things that she wants to do, like snowboard, and rock climb, and downhill ski and... what was she asked

Interviewer: Rock climb?

Jennifer: Rock climb. And she asked [the physician] last week if she could rollerblade. And she’s so onto the whole quality of life. She’ll say ‘Yes but it will improve my quality of life’. [...]And so as far as quality of life goes, we don’t even know what tomorrow will bring for
Charlotte. So having the opportunity for her to be able to snowboard and seeing her smile on her face.

There are certainly sports not appropriate with the presence of a CVL and parents discussed the need for saying no when necessary and also being prepared should something happen during the activities that they participate in.

Jennifer: She plays soccer. She wanted to take up rugby. I had to put my foot down. She did karate. So <laughing> you know, with this CVL, and all these tubes...I’ve learned to turn my back and let her go. As long as I don’t see it, I’m okay with it. And I always have 2 sets of clamps in my pocket ... <other parents laughing> just in case.

Unfortunately after years of experience, parents have realized that besides issues with central line safety, there are organizations that are not comfortable taking children with these medical complexities without the family providing a private nurse. There were missed opportunities due to their medical intricacies and the discomfort of others in feeling responsible for these children.

Jennifer: So she can’t go to camp. It’s not an option. Right, as many times as I looked into it. I’m talking about overnight or camp away, I’m not talking about science camp. Even going to science camp is complicated.

5.5.3.6.4 School

School can be a wonderful place for the children to integrate and experience a sense of normalcy. Michael and Kim mentioned that they would be sending their son, Noah, to the same school and class as his sister who was 10 months older than him to prevent him from feeling different.

Kim: It’s finding a balance, and making him feel that he’s not different from the other children. Um in terms of school for example, we’ll put him into a school with his sister, they’re going to be starting at the same time, they’re 10 months apart.

Some children had longer infusion rates and other devices (such as ostomies, drains etc.) or had not been toilet trained due to intestinal failure. As a result, there were complications in children attending regular schools. An additional challenge is that the school had to have a location to perform sterile procedures, if the PN was infused during school hours.

Jennifer: And that was another one that we kind of passed over, was being at school on TPN, is a big issue, right. Cause Charlotte is not invited to any school that we’ve dealt with so far, with all of her stuff, without a nurse. Even to go to school, to unhook Charlotte from her TPN, we have to find a proper room to do it in. You can’t just do it in the washroom, schools don’t have nursing rooms anymore.

Jennifer found that an alternative school was beneficial in having Charlotte feel accepted and integrated more easily. It was noted however, that it was essential to educate teachers so that
they were aware of the child’s chronic illness and challenges including coping with multiple absences. Many of these children experienced multiple hospital admissions for various reasons and as a result, missed school. In addition, it was helpful for the classmates to be educated in order to understand the reasons why they may see their classmate as different from themselves.

*Jennifer:* Well, you know what she actually goes to an alternative school, and they think much differently, sort of outside of the box. So the other children have accepted her better at this school than at her previous school. And she just sort of marches in with, she went in and we did an initial, ‘This is what all these things are, and this is who the nurse is, and this is what, why...’, in a kid-friendly grade 2 sort of way, with the help of her teacher. And there have been a few issues, because she misses so much school. [...] So, but even keeping up academically has been a struggle. And so her teacher has been extremely helpful in guiding us through that, and when we’re not there, making sure to talk about Charlotte a bit more. [...] But when she goes to school with her tubes and her stuff and her nurse, they just kind of ignore it now. It’s sort of Charlotte, and ‘thank goodness she’s at school today’ is the attitude.

However, despite all of the challenges and roadblocks that were faced in achieving this goal for Charlotte, Jennifer described how wonderful it was to see her daughter become part of this normal environment with other children her age.

*Jennifer:* And she had never gone to school, so seeing her being able to put her backpack on her back, as complicated as it is, and to be able to be in her school play last week for the first time ever, was so rewarding.

### 5.5.3.6.5 Friendship

These children have long-term hospital admissions and engage in discussions surrounding their illness and treatment plans. Growing up, they tend to have multiple interactions with adults, including doctors, nurses and allied health care professionals. For some children, this had an impact on their ability to relate to other children.

*Jennifer:* With friendship, developing friendship she doesn’t really know how to do that well. She could develop a friendship with an adult easier than with another [child her age].

### 5.5.3.7 Communication

When children were sent home on PN, there were nurse practitioners, nurses and a dietitian that provided support during regular working hours. The families had access to these individuals via email and telephone. The multidisciplinary team then connected with the medically responsible physicians regarding the children on home PN. The team knew these children very well and followed them closely from both clinical and social aspects. Despite a very supportive and
knowledgeable team, there was communication challenges described that occurred in terms of timing of response, evening and weekend care and during visits to the emergency department.

5.5.3.7.1 Outpatient Challenges

The transition from inpatient to outpatient was described as being challenging according to these families in terms of the speed of response to medical concerns. When their children were admitted in hospital, the families had daily contact with the medical team and their concerns were addressed, if not completely, at least partially on a daily basis. However, as an outpatient the response time was thought to be significantly delayed due to lack of daily rounding and the need for the staff to triage medical concerns on the basis of urgency. All of the parents agreed that this was one of the biggest challenges related to being an outpatient especially given the seriousness of many of their concerns. The parents described scenarios that portrayed these concerns.

**Jana:** I would say that is the most difficult part about having him on TPN for [my husband] and I. I think when we were living in hospital, although I never, ever want to go back to that place, if we had an issue, someone came in and did blood work right away. Or if we weighed him and he had dropped a significant amount of weight that day, the team would be in or a member of the team would be in and we would discuss it. Within a few hours we would have an answer or a game plan for the next morning. But living at home, it could take a few days to get an answer. Or someone’s out of town, so the whole team has to kind of email back and forth.

**Jennifer:** Well, you know, because if we had, if we were in hospital, we would have the answer to that extremely complex question that day, right. Typically either from a nurse, or somebody would come along and at least give their opinion. Typically a fellow or a resident or somebody that could at least ease our mind. But at home, and we’re concerned about things like line infections, blood clots, um fevers, which you know could turn ugly very quickly.

Jennifer elaborated on Jana’s example of weight loss which may or may not be dealt with in between appointments depending on the child, their nutritional and clinical status.

**Jennifer:** It makes it more stressful. Because I think, you know, things that perhaps, or even weight loss, for example. Weight loss is always an issue, always a problem, always a concern. So we’re watching the scales change right? But we’re not always where we want to be. So we wait 4 weeks for our next appointment to really hash it out at the table. 4 weeks of worrying and stress and concern and feeling like you don’t really have anybody who really understands, is so complicated.

The difficulty expressed by the parents is that they were at home worried that they may be missing something and fearing that their child may be getting sicker by not taking action. They
also expressed the fact that they possibly felt more concern than the health care professionals because it was their child.

Jennifer: And the answers don’t seem to come as quickly as we would like them to some days. But we are at home, concerned about our child. And if we ask a question, typically we’ve been through quite a bit. It’s because we’re very concerned about that. And I find that communication in ambulatory care extremely complex.

Erin: But it’s been an extreme stress. And sometimes I think, the other end, that’s you’re...it’s not their child, it’s your job. And obviously we worry more about our child than anyone else. But when you’ve been through so much and you know that that fever, that blocked line can mean so many life-threatening things, and you don’t get an answer that day. Like you write the email at 8 o’clock in the morning, and it comes 8 o’clock at night, and no-one’s...And nothing’s changed on her end, and you’re watching her, and thinking, I can’t go to the ER, we can’t get in the car and go to Toronto and just show up there. So like, what are we going to do with this? Then you’re going through, and you know, what if there’s bacteria in the line and we don’t know and it’s growing, you know.... You’re not sleeping well because you’re so worried. And then you send another email and you don’t hear, you call, and you’re, you know. And you’re just thinking, like I know they’re extremely busy and there’s lots of people with children like mine that they’re dealing with every day. But I’m the one at home going ‘what if I’m missing something, what if this is a (unclear). I should be in Toronto. Well, do I take the risk to go to ER? ’ I don’t have anyone that I can call and talk to, directly. That’s the negative of the parenteral nutrition at home versus the hospital.

In addition, many of these children were so medically complex that there were multiple teams that carried responsibility for different aspects of their care. These families expressed that at times they were uncertain who should be taking responsibility for each aspect and felt as though the buck was being passed.

Jennifer: [...] is that I find the communication extremely difficult, um at home to the hospital. So, and what I mean by that is not specifically with the GIFT team, but with the many other teams involved. So it becomes a bit complicated with who’s responsible for what, who’s going to take responsibility for this? It is an issue. Where do we go with the issue.

Jana: I’m nodding too. I’m nodding too.

Jennifer: And as the kids go, like I see Charlotte go home, each time with more and more and more complicated things. Like she has a G-tube, she has a J-tube, she has a CVL. Then the questions become a bit more complex.

There was also consensus regarding the quality of care received from the home PN support team when they were able to have a discussion of what the medical plan was going to be.

Jana: I really do find the communication is such a key issue for us. Because when you do get to talk to someone from the team, it’s wonderful. You finally have a voice of someone who understands [...]
Michael: But there have been times where I’ve been able to page [the nurses]. And we’ve gotten a call back right away. Sometimes it’s very simple, ‘Okay, you know what? Don’t take the chance. I’ll see you here in a hour.’ And that’s just what it is. So um we’re very, very fortunate in the sense that we are close, and there are a lot of times just being able to speak to [the nurse] has calmed us down quite a bit.

Interviewer: But that’s not necessarily the experience on the weekends...

Kim: Yeah, just between the hours of 8 and 4!

Michelle: And it never happens then.

Erin: Our kids don’t follow the 8-4 schedule. But yes, there are lots of conversations that are great [...]}

The parents expressed that inevitably, the children always became unwell when they were unable to converse with the individuals who knew their children best. They found this extremely stressful because the alternative was visiting the emergency department.

Jana: Um I find that Lucas, Murphy’s Law, something goes wrong on a Friday night or Saturday morning.

Erin: Always <all parents nodding in agreement>

Jana: And no-one is available. So many times we’ve said it would be so nice if, even if someone was on call over the weekend because we do the exact same thing – you play the game in your mind: if we wait a couple of hours to go to the ER, is this going to be life-threatening? Are we going, we live over an hour away, so if we drive down and it’s nothing, you know, that is a whole upset in Lucas’s schedule and his day. Just packing to even go down to the ER sometimes takes an hour or a couple of hours to get the meals ready for Lucas, to get all his supplies ready, to get a regular diaper bag packed.

5.5.3.7.2 Going to Emergency

When it was recommended or recognized that the child required a trip to the emergency department, the parents reported experiencing significant angst and concern. These discussions elicited strong emotional responses and a desire to change the system for the safety of their children.

Jennifer: Like when you have a concern, we don’t want to go to the ER. You know, that makes it even more complicated. And, I think that also puts us in another dangerous situation, where we’re avoiding the ER, but our kid might have something of concern. But rather than wait another day or another 2 hours or 4 hours or 4 weeks...um...

Jana: I’m nodding so much that my head hurts.

The concerns ranged from acquiring infections to the frustration of having to repeat their child’s long medical history to individuals who were unfamiliar with intestinal failure and home PN.

Jana: Because when you do get to talk to someone from the team, it’s wonderful. You finally have a voice of someone who understands, whereas if you call the on-call fellow or the...whoever happens to be in the ER that night, you have to kind of go over his whole novel of a medical history. And he’s only a year old, so I can’t imagine for those of you who have much, much older children. You have to retell his story every time.
Erin described the fear and frustration she felt about the emergency department because of a past negative experience.

**Erin:** Oh, the ER room scares me. My daughter’s almost died of a gastro virus already, like literally, in my arms, um which she caught in hospital, not this one. Someone else brought in, it wasn’t us. We got sick after her. So I’m paranoid to go to the ER. If she’s not sick already, she will be by the time we leave, and they don’t understand her. So it is a frustration sometimes.

Michael also reiterated the frustration related to having to retell his son’s medical history during emergency visits.

**Michael:** But when you come, it is a little frustrating. Sometimes you’ll sit there for hours. And you’ll say, ‘This is the situation with Noah,’ and you just sit there. And you wait and you wait and you wait. And you have to, I mean, Kim’s better at it than me. But then to retell the story of, you know they’ll say, ‘Okay, what’s his history?’ and then...
**Kim:** You would think it would be in the system.
**Michelle:** You just want to record it. Here listen to this. Lots of agreement (Unclear overlap by several people).
**Erin:** (Unclear)….have to tell the story over and over.

It was evident that there had been a lot of frustration related to visits to the emergency department although Michelle expressed that at times it was a necessity in order to gain piece of mind, it was a necessity. Her daughter also had multiple admissions and she felt, as opposed to the other parents, that the staff understood her daughter’s clinical situation.

**Michelle:** We hate it, we hate going there, but, got to suck it up if it means always, like 11 at night, so we’re there during the night.
**Erin:** On a Friday night
**Michelle:** Of the long weekend. But if it means peace of mind and Alicia’s okay, whatever. We’ll lose some sleep. And they are usually pretty good. Most people know her, so in all her complexities. And that’s just the way it is.

5.5.3.8 Intestinal Failure

Intestinal failure is an uncommon disease and long-term home PN, an uncommon therapy, of which many of these families had not heard of until their child was diagnosed. There are challenges for parents of children that have a rare disease in that health care practitioners may not be familiar with the condition. Intestinal failure initially results in long-term hospitalizations in order to allow the patient to experience intestinal adaptation and stabilize metabolically and nutritionally in order to send the family on a home PN regimen. Decisions regarding the child’s medical therapy can impact their QOL and parents are mindful of this. All of these issues were presented in discussion by the parents in the focus group.
5.5.3.8.1 Diagnoses

There were differences between families in their experience of having their child diagnosed with intestinal failure. Within the focus group, some families expressed that they were aware when the baby was in utero, others experienced a delay in diagnosis over a year and others experienced a sudden medical event causing intestinal failure and resulting in home PN therapy.

Michelle: That was from the day she was born she was on TPN. She had gastroschisis. So we knew even when she was inside me what we were looking forward to. So she was on it from day 1.

Erin: When I first found out about it, I had never heard of it. Like I... we, our stories, Abby was preemie – I’ll just do it really briefly – so we had a preemie who ended up being in the hospital for a few weeks. I knew something was wrong, the neonatal... whatever, she’s fine, they were too busy. And I had a bad recovery so I didn’t really click until I got home why her bum was so sore, was because she was pooping constantly. And they weren’t recognizing that. So I cried the first night we took her home because I’m like, there’s something wrong. [My husband’s] like, ‘No, they said she was fine.’ I don’t care what they said, I know something’s wrong. Babies don’t poop this much. So then she spent a month in hospital. Like we had her home for 9 days. She was in the hospital for a month, then home for a week, and in for a couple of weeks. But then we had her home for almost a year. And she was tiny, but we were getting by, and you know. And then we got told she was too tiny, and then the nightmare began. That we had to admit her to be NG-fed. And the nightmare began when they started over-feeding her, and her bowels just can’t handle that [...] Um, the first time it [parenteral nutrition] was mentioned was a few weeks before it actually happened. I think there was some denial that my child needed it: ‘No, you know, like she’s tiny. So what? If you don’t over-feed her like you’re insisting she needs fed, she’s growing, just slow. Like, she can be little’. So I think I had to face some denial that Abby needed that. Then by the time she got her first CVL, we would have lost her if we didn’t have it.

Kim: [...] Noah wasn’t always sick.
Michael: Just the fact when I had to bring Noah to the hospital. When he was, I say ballooned up, we didn’t know what it was. And then when we first met [the doctor], and he explained what happened, that was enough of a shock, when we’re now going through, okay, what do we have to do to get Noah... how do I say it, for him to recover...

Erin felt that due to a lack of knowledge and understanding of her daughter’s clinical state, the physicians caused a delay in her diagnosis. In the meantime, Erin felt that Abby became more unwell because the medical team was setting unrealistic nutritional expectations. Erin expressed frustration in the longstanding issues that she was now facing due to, in her perception, the original treatment that was attempted.

Erin: But unfortunately we met some people that weren’t willing to face that they didn’t understand Abby, and just kept doing the same thing, and dug the hole deeper, for us. So she doesn’t eat at all orally now, because her bottle was taken away from her, that’s just one example. And they put her first CVL in, which completely clotted in less than 2 weeks, and she was already very sick and was sicker. Um and when we were faced with that first CVL, we’d been digging this hole and they weren’t listening to us. And we got to that point they said absolutely she could not go home, while she was on, while she had the CVL. And told us it would be at least
There was general consensus amongst the families that most of the doctors they had dealt with on a consultant basis e.g. Emergency, had a lack of understanding of their child’s chronic illness and had to be convinced about the possibilities of what may have been happening clinically.

**Erin:** Okay, we can’t, and Abby’s so complicated that even to show up in the ER here, which we did, thank goodness [the home PN nurse practitioner] was there. Like she just has things that doctors don’t believe you. Abby doesn’t go by the book.

**Jana:** You have to retell his story every time. You have to you know kind of explain, no, he doesn’t do everything in the typical way. Yeah, this totally could be happening, even though you’ve never seen it in any other child before. Yeah, I find it very frustrating.

**Jennifer:** Because our kids don’t do things the way they’re supposed to or you know.

**Erin:** Throw the book out.

### 5.5.3.8.2 Long Term Hospitalization

Prior to discharge home on PN there was a period of long-term hospitalization in which many therapies were attempted and finally the home PN teaching occurred. This period of time was recalled by these parents as a significant period of stress on themselves, their children and their families. Michael and Kim remember how difficult it was that their family was apart.

**Michael:** When Noah was in the hospital, Kim was at the hospital with him, so I was at home with the other children. So all of that on me was a lot, as well as the mental stress of just being there and dealing with Kim. Not to mention, we’re split apart. Um, so that was...that was very hard. It seemed that everything that could go wrong during that period went wrong.

Long term hospitalization can impact development as well and Erin described her experience with this aspect once she was discharged home.

**Erin:** And being home, I mean, she was so weak, that she couldn’t even stand up when we left here. And that wasn’t because, she hadn’t been that, it was a hospital symptom.

### 5.5.3.8.3 Uncertainty of PN Side Effects

The parents were all cognizant of the potential side effects of long term PN. Each parent seemed to have their own specific concerns, all which were valid and documented side effects of PN.

The uncertainty surrounding the occurrence of the side effects was an expressed source of stress
for the parents. Erin and Kim discuss their concerns about the side effects of PN on their children.

**Erin:** One day at a time. Like it’s something I think you need to do when you have a child that’s sort of, is living with issues that could change tomorrow. You know, we could have an infection tomorrow.

**Kim:** The main thing that scares me is the damage that it could potentially have on the liver. That was huge but anyways you have to look at, well it is saving his life.

Erin also attempted to put it into context, and that no matter what all of the uncertainties were, it was important to try to focus on enjoying life and family.

**Erin:** It’s still scary. I mean, none of us are ever free of that worry of them becoming septic or clots or whatever. But you can put that at the back of your mind and enjoy being a family.

### 5.5.3.8.4 Medical Impact on QOL

Many of the medical implications of intestinal failure and home PN have had a direct impact on these children’s QOL. Erin described the decision-making process that she and her husband faced regarding the type of central line to choose for Abby, given the consequences she faced with her first type of intravenous access.

**Erin:** But to maximize, and one example even just right now: she’s got to get her CVL rewired or fixed, hopefully, fingers crossed and not a new one, and we’ve had a big discussion about PICC versus you know which type, because we’re very concerned because when she’s had IVs in the past, she wouldn’t use that arm. It’s like, gone, to her. So now we’re faced with the possibility of a PICC, we’re worried that her quality of life as an almost 3 year old is going to go down, because is she going to use her arm? Are we going to need physio again, because she thinks she’s not – well we don’t know until we try it. And hopefully we don’t have to. But you’re faced...and that was a big discussion that [my husband] and I had, like her quality of life, we want it to be the best it can be, for a 3 year-old, in her case. She needs her CVL, but, you know we’ve tossed, should we ask them to put another one in her neck? We know that there’s risks, there’s negatives, but if she doesn’t use her arm for the next 4 or 5 months, then we have other problems.

As previously discussed, symptoms that would have required minimal workup in a healthy child resulted in an extensive workup for these children. As Jana described, she believes this also impacted her son’s QOL.

**Jana:** But for example, Lucas had an ear infection last week, and went to the hospital, went to the ER, spent the whole night in the ER until they could figure out what was going on, had blood work done from his PICC line, had blood work done from his hand. [...] And then he was put on antibiotics, and of course, the antibiotics did a number on his gut. And he’s been super sick on top of having an ear infection. So what maybe would have taken 1 or 2 days to get under control for a typical guy, ends up being a 3-week event for Lucas, so it sort of changes his quality of life in that regard.
5.5.3.8.5  “Not a checkbox disease”

Intestinal failure can be the resulting diagnosis from a number of different diseases. Parents described that because of this, the children had not qualified for additional funding nor for special activities that were disease-specific. Jennifer spoke about this in length.

Jennifer: And that’s the other thing is that I think it’s a bit isolating for Charlotte, because she can’t go to camp (unclear), she can’t go to transplant camp, there’s no GIFT camp. She doesn’t really fit, she’s not ticked off in any little box anywhere. And so any financial or any, everything is a struggle. We have to write all kinds of crazy letters, and you know she’s not incontinent, but she kind of is because of the TPN. But that doesn’t fit the incontinence grant. We also don’t fit any checked box. So we can’t, we’re kind of a caregiver, but we’re not really a caregiver because it’s not an older person. Like, they’re not really incontinent, but they kind of are. Are they disabled—kind of, depending on how you define...so we’re always playing with definitions. And you know, you all have to do some fancy footwork for letters, and you know there are really no programs for, in our case, for Charlotte.

5.5.3.9  Lessons Learned

The parents discussed the aspects of having a child on home PN and how this had impacted their lives. They expressed that having a child with a chronic illness required a re-evaluation of their expectations, an acceptance of the loss of health, learning to move forward and felt that it put different aspects of life into perspective.

5.5.3.9.1  Letting Go of Expectations

Before a child is born, many parents have imagined things that they would do with their children. Jana discussed the experience of having to let go of or at least delay some of your expectations when your child is born unhealthy.

Jana: I think when you have a child who’s born with any kind of chronic illness you go through, not a grieving process, but you sort of have to let go of some of the things that you had expected to do with that child. And maybe it all eventually happens, or maybe it will happen in a different way, but you just have to kind of think outside of the box and go with the flow.

5.5.3.9.2  Not a Choice

There was significant reference to maintaining positivity and accepting or adapting to try to be positive and to adjust and accept the situation of having a child on home PN. These coping mechanisms were evident in many aspects of discussion. Despite this, it was also noted that going home on PN, living life with intestinal failure, and having to make continuous adjustments
to family life were not a choice that anyone would make. Erin mentioned this directly on two separate occasions.

**Erin:** How will it do, what can we do to make this work for our family. And luckily her siblings are very good about it. Nobody’s asked for this.

**Erin:** Once we got here and it was, ‘You know what, we can train you to do it,’ you’re not happy, nobody chooses this. We’d all rather have no machines and no tubes. You know, I used to think that kids just learn to eat. Now I have one that won’t eat. You know, all those things, we’d rather not.

The same concept was evident when it came to the decision to put their child on PN and to go home with this therapy. These treatments were keeping their children alive.

**Erin:** And then she got a gastro in the hospital, and the same thing. You know, ‘You’re holding your little girl for the last time, or you agree to let her go get a CVL.’ So it wasn’t a choice. It was something we had to do.

**Kim:** ...but anyways you have to look at, well it is saving his life. And so it really isn’t a choice. It’s something that you just have to do.

### 5.5.3.9.3 One Day at a Time

As previously mentioned, there was a sense that some parents often felt they were always one step away from a complication or a negative change in clinical status. This seemed to have resulted in the parents living in a ‘one day at a time’ mindset. The message they portrayed was that they enjoy what they have for the moment, since tomorrow may be different.

**Jennifer:** So you know...but tomorrow will be different. So in our home, I think that changes every single, minute to minute really, we don’t know what the next 5 minutes will bring.

**Erin:** One day at a time. Like it’s something I think you need to do when you have a child that’s sort of, is living with issues that could change tomorrow. You know, we could have an infection tomorrow.

### 5.5.3.9.4 Appreciation

The one day at a time mindset led to another concept that parents discussed, which was an appreciation for small things in life. They suspected that other families take things for granted because they were common events. They attributed this to having had the experience of a child with chronic illness and for some, to almost having lost their child. They also had an awareness of knowing things may change quickly. Both Erin and Jana perceived that their children had great QOL despite their challenges because of the fact that they, as a family, appreciated small successes in a day more than others.
Erin: But her quality of life in some ways, I think, has actually become better than some of the families that I know that have no sick children in their house, because we appreciate things that we wouldn’t have, you know. They’re just taking everything for granted when we’re like, ‘Wow, we had a great day today, as a family.’

Interviewer: So a great day would mean…

Erin: Even going to the park together, having a picnic or you know, whatever. Just things that other people just…going for a bike ride. People think, ‘Oh yeah, of course you do that.’ But when we have a good day, Abby’s feeling good, everybody’s happy, there’s a different appreciation there I think, which is something to be thankful for in this world of chronically-ill children.

Jana: I think Lucas has a great quality of life. I think I definitely agree with the mom who said that you tend to appreciate things more. A lot of the small things that my girlfriends do with their kids they… I think they take for granted. And we probably would have done the same, had we had a child with no issues.

5.5.3.9.5 A Person, Not a Diagnosis

Families truly appreciated a medical team that treated their child as a little person rather than a disease. It was this concept that should encourage medical teams to focus on how to normalize life for these children and to understand that medical decisions need to be made in the context of preserving a good QOL for these children. This was very important to these families.

Erin: [...] she’s a very active little girl. And if you cover up her CVL, and didn’t know, and thought she… you know, you might think in some circumstances, that she had no issues…So the positive for us is that we do have a team that recognizes that there are other aspects of Abby besides her CVL, and needing parenteral nutrition, that she is a little girl that has other needs.

Erin expressed that the medical team that had been following her daughter had a sincere appreciation of Abby as a person.

Erin: So I think the [medical] team, more than any other physicians that I’ve met, they just realize that it’s a quality of life, and our little people, are little people…not just little people but, you know, not their health issues, they are people that need to be able to be the best person and the most free to be them that they possibly can be.

5.5.3.10 Gratitude and Hope

Individuals with intestinal failure are dependent upon PN for survival. Parenteral nutrition provides calories, nutrients, fluid, and electrolytes to maintain hydration and nutritional status and thus growth and development for these children. Without PN therapy, these children would not have survived. The parents expressed their gratitude for the availability of the therapy as well as their ability to provide this therapy at home rather than being hospital-bound. In addition, they were grateful for a medical team that understood and followed their children so
closely. Although, each expressed hope that one day their child would be able to sustain life without dependence on this technology.

5.5.3.10.1 Availability of Therapy

Parenteral nutrition has prevented the deaths of children with intestinal failure who would have otherwise been unable to sustain their fluid, metabolic and nutritional status from enteral feeding alone. Both Erin and Kim expressed their gratitude for the availability of this therapy.

Erin: But, we have her, she’s doing well in lots of ways. You know, look at what you have instead of what you don’t have...Yes, we have our scary moments and whatever, but every single person here is just thankful to be home, and thankful that they have their child, which 30 years ago we wouldn’t have.

Kim: [...] you know we’re lucky to even have this opportunity, because 30 years ago it wasn’t even available and the children would die.

5.5.3.10.2 Being at Home

In addition, the parents appreciated that many therapies would require sustained admission or frequent visits to the hospital and they were very grateful for the opportunity to be able to have their children at home. Also, they were aware that many other hospitals do not have a home PN program to support discharge of these patients to the community. The ability to live as a family was expressed to be of immeasurable importance, despite the challenges that they face related to home PN.

Michelle: That was from the day she was born she was on TPN. She had gastroschisis. So we knew even when she was inside me what we were looking forward to. So she was on it from day 1. And the nice thing was that we could take her home on it. So we were in the hospital about 6 months. Then we got trained so [the doctor] was like, ‘Well, improve your quality of life, you can do it at home.’ That’s great. So for us it was an easy transition. [...] Again, we’re just thankful she’s home.

Erin: [...] So the plus for us is that it allows us to be home and be a family. And that is huge, as we all know; I’m sure, more than anybody else, in a normal situation, quote, unquote. So there’s positive part, of it, I think, is I’ve seen that there are people that have children like ours, if you will, that are living in the hospital, where we are allowed to be home.

Jennifer: I mean, her quality of life at this point in time, as complex as it is, is so much better than having to live in the hospital... So everything is great. Thank God we’re home, and I wouldn’t want to trade that in for the world, because living in the hospital for 8 or 9 months at a time sucks! <laughing>

Erin: More than that.

Jennifer: That’s probably the only word I can use!
Erin spoke about her initial experience in finding out that her child would require PN in an institution that did not have a program to support the provision of PN at home. She also discussed the difference in their emotions of having a child on PN once they were told they could go home with the therapy.

**Erin:** So after a lot of hard time there and knowing, like facing that – I’ll never forget how you feel when you’re just being told that your child will not go home on it [parenteral nutrition]. We didn’t understand, we were new at this game, if you can call it a game. So when that first time, one of the doctors said to us, ‘You know, well sometimes we just put in…put in an IV.’ I was like ‘What! What do you mean?’ Because there, it was also linked with, if this happens, you live at the hospital until she doesn’t need it anymore. So it was, what about my other kids? And also with me, like with us, when it was told, it was told in a way that ‘you are never going home, until she doesn’t need it’. So that was, we panicked, basically. Like, you know our family won’t be together. And I think that’s what it is. Once you get used to the idea, and you realize that this way she can be home, like once… that was a whole different perspective. Once we got here and it was, ‘You know what, we can train you to do it,’ ‘you’re not happy…but it’s a whole different feeling when it comes with ‘we can train you’, you know. [...] So I think it’s a whole different thing when you’re given that option, as in we can do this at home; and the option as it will keep them alive but it’s in the hospital treatment. So the whole home TPN program just allows a quality of life that none of us would have the opportunity to have, if it weren’t something that we could be trained to do at home.

5.5.3.10.3 **Medical Team Understanding**

Having a medical team that understood the experiences of having a child on home PN and their children’s chronic illness was a very important aspect. This was also addressed previously in the theme of Communication. The parents emphasized their gratitude of recognizing their children as little people, the fact that the team has provided understanding and guidance in difficult situations and also that they were always trying to improve their services within the constraints of funding and available personnel.

**Jana:** [...] Because when you do get to talk to someone from the team, it’s wonderful. You finally have a voice of someone who understands...

**Erin:** Honestly the feeling that [my husband] and I, and even Abby, even our son feel when we come is just, we’re safe here, the people care here. And it means a lot. And I think you’ve been trying to improve. And they’re listening, and thinking, okay, this is where the parents are, this is what…then it will. [...] And I don’t know the others, but just to think, like you know what, if there’s a team working with us, that’s trying to help us, but also trying to help the people that are coming behind us, you want that too. You don’t want anyone to have to face some of the scary things we have, and just trying to increase the support is, in itself a great thing.
5.5.3.10.4  Discontinuation of PN

Each family expressed the hope that their child would come off of PN someday. Kim described her experience of coming home on PN for the first time and meeting someone whose child had been on PN for a very long time. This was a very negative experience for her because it instilled the possibility that PN would be permanent for Noah.

*Kim:* I have talked to, one of the first nurses that came in when Noah came home, her daughter was on TPN for 21 years. And...and that scared me. Because in my mind I’m hopeful that Noah’s going to outgrow this.

Erin and her husband have kept their trailer in hopes that they will once again be able to use it providing Abby comes off of PN.

*Erin:* We used to camp. We have a trailer we haven’t used for 2 years, because we can’t go camping with the CVL. But yet we haven’t sold the trailer because we hope that someday we’ll be able to use it again, and they [siblings] know that.

*Interviewer:* Don’t, don’t sell it.

*Erin:* And we’re not!

Michelle also expressed her hope that her daughter would come off PN one day but appreciated that there was a chance that this would not happen.

*Michele:* We hoped one day she would not be on TPN. I don’t know if that will happen. But um to us it was, that’s what keeps her alive. So that’s just what it is.

5.5.3.11 Generic Survey Discussion

At the close of the focus group, there was a short discussion regarding the use of the generic surveys for the quantitative aspect of this study. Parents were asked what their opinions were concerning the surveys. The overwhelming response was that they were *too generic.* In order to clarify, Jennifer commented:

*Jennifer:* It would be nice if there were something specific to home TPN <looking at survey> because pain, well pain, like, what kind of pain? Pain that they feel when they flush, or the pain of an operation, or the pain of an obstruction, or whatever the case may have been that they initially had.

Another example provided by Jennifer illustrated that many of the questions could be interpreted different ways because of the complexities of care and the struggles of children on home PN.

*Jennifer:* But even like this question here <reading from survey>: ‘walking or climbing a flight of stairs’, well, with or without an IV pole. It’s really different thinking.
Erin expressed concern that she worried the researchers wouldn’t truly know what she was trying to express because the questions were non-specific.

**Erin**: Same thing. Too generic. I think when I was doing it, I did my best to answer it, but I was also thinking, I don’t know what [the researcher assistant’s] gonna get from this. [...] I tried to do key little notes, but it was so...generic, I think, that I’m like, I hope she gets what she wants. But as I mailed it away, I was sort of thinking, does she [the research assistant] really know where I’m coming from?
Chapter 6
Discussion

6.1 Introduction

A mixed methods analysis was utilized in this study to describe QOL in pediatric patients with intestinal failure on home PN. A battery assessment of quantitative questionnaires was administered in order to obtain a multidimensional view of QOL. The results of the questionnaires completed by the home PN sample were compared to published normal populations where available, published SBTx data and to a small population of SBTx patients from the same institution. A focus group was conducted with parent proxy representatives for the home PN population. The combination of the quantitative and qualitative approaches allowed for a comparison to relevant populations, including healthy children and children post-SBTx and facilitated the acquisition of insight into the lives of patients on home PN and their families. In addition, the qualitative component was chosen to assist with the interpretation of the quantitative results. This discussion will centre on the multidimensional aspects of QOL including physical, emotional, social, and school functioning as well as overall QOL, general health and impact on caregivers and family. The results from the study’s qualitative component will be further described as a summary of the proxy-described lived experience on home PN.

6.2 Key Findings

Proxy assessment of children on home PN in this study established their perception that these children were different compared to the normative population in domains including physical, social, emotional, and school functioning, general and overall health. The parents perceived that their children experienced higher levels of pain than healthy children and those who were post-SBTx. The parents scored their children’s QOL less than the healthy cohort however in the focus groups expressed that they were satisfied with their QOL considering their need for home PN. Having a child on home PN had a significant impact on the family and siblings as well as the primary caregiver in terms of limiting activities and imposing isolation. The primary caregivers were at risk for fatigue, isolation and poor QOL. Alternatively in the comparisons to the SBTx population, the individual scale assessment and overall assessment of QOL were very
similar. However, more proxies of children post SBTx believed that their children had a high versus low QOL and experienced less bodily pain.

The children on home PN rated themselves similar to the normative sample in many aspects however there were differences in their functioning from a physical and psychosocial assessment standpoint. These children also rated their general health lower than the healthy children and children post SBTx, both currently and in the future. Other than this rating of worsened general health, the home PN patients scored themselves comparably to the SBTx population.

6.3 Battery Assessment

The tools chosen for the battery assessment of multidimensional QOL were reviewed in terms of their psychometric properties to appreciate their strengths and limitations.

The PedsQL™ Generic Core Scales (Proxy and Self Reports) as well as the CHQ Proxy and Child reports and ITQOL were extensively studied in terms of reliability and validity however provided minimal detail regarding the item generation and tool development phase in terms of sample sizes and detailed methodological designs. They had significant normative population data available for comparison, most of which was American or Dutch. There was one comparison from Canada available for the ITQOL however it encompassed only one age group, preschool children and thus was not selected for use\(^\text{108}\). Unlike the CHQ and ITQOL questionnaires, the PedsQL™ had the advantage of fewer items and reduced respondent burden as well as a total overall QOL score. This allowed for an overall quantitative assessment of QOL in addition to scores from subscales that Varni et al. assessed to be contributors to QOL.

Generic QOL tools are imperative for comparison to normative populations however they often miss important disease-specific issues, as noted by the parents in the qualitative focus groups. Some parents who completed the questionnaires wrote additional comments to try to explain their perspectives. During our focus group, one mother of a child on home PN expressed that she felt the generic survey did not allow her to make her concerns known and worried there would be little value for the researchers. There were no disease-specific questionnaires for the home PN or the SBTx populations available and thus the qualitative aspect was utilized to assist with the interpretation of quantitative findings.
The PedsQL™ Family Impact Module was chosen to assess the impact of having a child with home PN and post-SBTx on the families and caregivers. This was a relatively new tool and the only tool we were aware of that specifically assessed the impact of chronic illness on the parent and family functioning. This tool had minimal information published about its development and limited reliability and validity data. There was some evidence of construct validity, internal consistency reliability and test-retest reliability\textsuperscript{102, 151, 152}. At the time of the study, there was no available normative data for comparison.

The Functional Status II-R was used to measure function in relation to chronic illness in the children on home PN and SBTx as part of the multidimensional construct. This tool had been used in several studies and reliability and validity data was presented by the author for the long version however there was much less information related to the 14-item form used in this study thus limiting our assessment. The short form was chosen in order to reduce respondent burden as the generic QOL CHQ and ITQOL tools were quite long. There was published normative data available for comparison.

The VAS assessments were easy to use and had very low respondent burden. Most of the data supporting reliability and validity had been studied in pain and mood constructs. One small study looked at its use for QOL. In addition, it was difficult to interpret numerical differences in scale measures. However, these tools provided an overview of the specified constructs and contributed to the interpretation of scales on the generic tools.

Ceiling and floor effects were not a great concern in this study with only a small number of multi-item scales exhibiting a significant ceiling effect (>25%). One limitation of this assessment was the small sample size which may have contributed to higher percentages.

6.4 Comparison to Normative Populations

6.4.1 OVERALL QUALITY OF LIFE

Pediatric patients with intestinal failure on home PN scored significantly lower on various domains of generic QOL assessment tools as measured by parent proxy. The total QOL score on the PedsQL4.0™ was significantly lower than the published normative population, a difference of approximately 20 points. The other tools utilized, ITQOL and CHQ-PF50 did not
have summary scores for QOL comparison. These results were in contrast to Gottrand et al. who found that overall, children on home PN did not have a significantly different QOL compared to a normative population. There were, however, differences in the results by age and specific domains that were lower than the norms. Gottrand et al. also utilized different measurement tools than in this study and a French reference population which may have contributed to the difference in results.

The 10cm Visual Analogue Scales provided a global assessment of QOL over the past 3 months. Normative data is not available for comparison. On the overall QOL scale, only 2 of 13 parents scored their children greater than 8 cm which had been used in published research as an indicator of a high subjective QOL. The remaining 85% scored less than 8cm, which was considered a lower QOL. Of the 11 patients who rated their children less than 8cm, only 2 were between 6-8cm and all others were less than 6cm. A different cut-off may have resulted in a variation of these results.

Although the parents rated their children quantitatively as having a QOL less than that of the normal population, all of the parents articulated in the focus groups that their children had a good QOL despite being on home PN with intestinal failure. This may have been associated with their description of QOL as a relative concept: “If... quality can be relative, relative to what you’ve experienced”. The low results on the PedsQL total QOL summary score may have been related to the awareness that the children were unable to have and experience all that other healthy children could. However, during the discussions, the parents expressed satisfaction with the fact that their children were able to live and enjoy life at home with their family and were able to engage in many normal age-appropriate activities, within their limitations. There was discussion regarding the desire for normalcy, but normal was expressed within the context of their lives. Parents relayed having redefined normality in their family and had been attempting to adapt within their new concept of normal. Quality of life for these parents was described as a dynamic concept that can change day to day. Thus the timing of these assessments would significantly impact the results of the measurement.

The children who provided self-ratings for overall QOL on the VAS (n=4) and the PedsQL4.0 (n=6) had variable underlying diseases resulting in differences in presence of stomas, need for
ental feeds, number of infusions required and duration on home PN. Overall, these children received PN infusions between 3 and 7 nights weekly, attended school, and had been on home PN ranging from 3 months to 17 years. A sample of 4 children on home PN self-rated their overall QOL on the 10cm VAS. There was a wide range of responses from 2.1 to 8.4cm, indicating very low to high scores. The one particular low rating of 2.1cm was that of an older child who was placed on PN within the last year. Six children (ages 8 to 18 years) on home PN completed the PedsQL™ self report and also demonstrated an overall significantly lower score in the total QOL score with a mean of 67.9 out of 100 (normal age-matched mean was 83.8), with a range from 45.7 to 77.2. Both the VAS QOL and the PedsQL™ self reports were indicative of the variable perceptions these children had about their QOL which may be due to the differences in underlying disease, home PN regimens and/or differing levels of morbidity. The sample size was inadequate to statistically assess the impact of these variables on QOL assessment. It was suggested by Gottrand et al. that children who had been on PN since they were very young learned to adapt in comparison to those started more recently. The adolescents in that study rated their QOL higher than those in the normative population which was attributed by the authors to having effective coping mechanisms allowing them to become accustomed to their lives. Gottrand et al. also stated that unlike adults, many of these children had never lived a healthy life for comparison. In our study sample, 2 of 6 children had been on PN since birth while the others were started later, potentially contributing to the variation in results.

The qualitative analysis by proxy indicated that parents ranked their children as having a lower QOL compared to published norms as well as lower scores on the VAS overall QOL scores. However, in the focus group discussion the parents expressed satisfaction with their child’s current QOL given the circumstances of their health and medical requirements. The self assessment by the children in this study demonstrated variable results on both the PedsQL4.0™ as well as the VAS. Overall they scored significantly lower on the quantitative survey compared to published norms.
6.4.2 GENERAL HEALTH DOMAINS

The domain of general health in the **proxy-completed** ITQOL and CHQ-PF50 asked the parents to reflect on their perception of their child’s health in the past, present and future. These scores were significantly lower than the published norms. The VAS also demonstrated a similar outcome with only 2 of 14 parents rating their child’s overall health over the past 3 months greater than 8cm, and the remaining 86% rated their child less than 8cm (considered less than optimal perceived overall health). This was consistent with the results by Gottrand et al. where the assessment of health was significantly lower across all age groups of children on home PN

It would be interesting to be able to delineate the results of the perception of health at different points of their child’s lives. Concerns of the past, present and the future were all mentioned during the focus groups. Many of these families indicated that they had experienced a time when they believed that their child may not have survived. They expressed that they currently needed to protect their child in order to prevent them from acquiring infections, since the impact was more severe than on a healthy child. Also conveyed was a sense that their children were always one step away from a hospital admission and a feeling of uncertainty of their child’s health status related to disease and PN-related complications, both in the present and future. All of these contributing factors promoted insecurity regarding their child’s health.

The children rated themselves significantly lower in the general health domain on the CHQ-CF87 with scores ranging from 18.8 to 44.6 out of 100 and the overall mean scores was 30 points less than norms. There was also a high variability in VAS ratings of overall health (rating over the past 3 months), from 0.8 to 9.7cm (median = 7.0 cm). The CHQ-CF87 scale which asked the kids to reflect on past, current and future health concerns, had less variable results than the VAS. One question on the CHQ-CF87 that may explain the lower results in comparison to the VAS was that the kids were asked to reflect on their health in comparison to their peers. The sense of being less healthy than their peers was consistent with Gottrand’s findings where the adolescents rated their health significantly lower than healthy children and may account for the difference in results in this study.

Both proxy and child ratings implied that the perceived general health of the child on home PN was significantly worse than that of the normal reference population.
6.4.3 PHYSICAL DOMAINS

Various aspects from the battery assessment will be discussed under the physical domains including overall physical function, energy and fatigue, bodily pain, growth and development, dietary restrictions and functional status.

The ITQOL, the CHQ-PF50 and the PedsQL™ proxy reports each demonstrated significantly lower scores on the physical functioning scales in the home PN sample in comparison to published norms. The physical summary scores on both the CHQ-PF50 and the PedsQL™ were also significantly lower. This exemplified a perception by proxy that over the previous month, the children on home PN had more difficulty participating in age-appropriate physical activities of daily living, recreational activities and had lower overall energy. In contrast, a small study from France looked at 11 children on home PN and found no significant difference in energy expenditure and physical activity in comparison to children matched for age, sex and body composition\textsuperscript{166}. Fatigue is a significant contributor to the impact of chronic illness and an important component of QOL\textsuperscript{167}. A study reviewing pediatric cancer survivors found that fatigue was the only factor studied that was related to both the physical and psychosocial summary scales on the PedsQL™\textsuperscript{168}. Gold et al. assessed fatigue and QOL in children and adolescents with chronic pain and found that there were moderate relationships between self and proxy reports of QOL\textsuperscript{167}. It was noteworthy that the parents in our focus group expressed that the children had significantly more energy once starting PN and when feeling well the children were able to participate in many activities, however satisfaction and benefit of these enhanced activity levels were not directly discussed. One parent wrote on the survey that their child was unable to play as long as other children due to the duration of the PN infusion. Some parents in the focus group commented that the survey questions on physical activity could be interpreted in many ways. As an example, the response to the question: \textit{Has your child been limited due to health problems with running?} would be dependent on whether they were connected to the IV pump or not or whether they were unwell at the time. In this study, according to parent proxy reports, it appeared that physical activities were limited due to having home PN with a central line, long PN infusions, as well as limitations in energy reserve.
The children who self-completed the surveys scored significantly lower on the PedsQL™ physical functioning scale, yet this was not evident on the comparable scale of the CHQ-CF87. The mean score difference on the PedsQL™ was 10 points and had a narrow range of scores, making it difficult to assess the true clinical significance. The differences between the items on the physical functioning scales on the 2 questionnaires were that the PedsQL™ asked two questions relating to hurting/aching and energy level whereas the CHQ-CF87 was solely activity-related. The addition of energy and pain aspects may have contributed to the differences in the scores. One study found the most common complaint from adult patients on home PN was fatigue. It was possible that the patients felt they were able to participate in most of the listed activities however they may not have possessed the stamina nor recovered as well following these activities. Similar to the results in this study, Gottrand et al. found that the adolescents in their study rated themselves significantly lower in terms of sports compared to their healthy peers. Conflicting results from a small study by Silver who interviewed 3 children (aged 8-10) on home PN and reported they were able to participate in similar activities as their peers.

There was a significant difference on the ITQOL proxy report in terms of bodily pain compared to the normative population however this difference was not present in the CHQ-PF50 and the PedsQL™ does not address pain separately. The VAS assessed overall pain in the past 3 months by parent proxy (0= most pain, 10= no pain) with 5 of 15 parents rating their children above 8cm (approaching no pain) and the other 10 parents rated their children below 8cm. The median score however was 7.5cm demonstrating that many parents felt that their children experienced above average amounts of pain but not severe pain. The ITQOL questionnaire related the question on pain to many different issues such as gas, teething, and injury/illness so it is difficult to interpret this significant difference in terms of home PN alone. Some parents wrote comments beside the pain items indicating that the child had a sore throat, had been teething and also recently had a foot cast. However, during the focus groups the parents did express that their children experience pain related to certain home PN procedures such as cap changes and flushing of the central line. This was consistent with a study by Engstrom et al. in Sweden where the parent proxy assessment indicated that their children complained of non-specific bodily pain. The self-ratings of pain on the VAS scale by 4 children on home PN were similar to the proxy ratings with a range of 5.3 to 9.1cm, however there was no significant difference on the bodily
pain scale on the CHQ-CF87 when compared to norms. This study indicated that there were some children that experienced pain related to home PN procedures and symptoms from underlying disease as rated by proxy and self-completion of surveys however the significant differences in the younger age groups were related to both PN and non-PN related concerns. Studies in children with chronic pain, juvenile arthritis and Spina Bifida have demonstrated that pain is an important predictor of overall QOL\(^{167,169-171}\). Pain can have a significant impact on QOL and should be reviewed further in this population.

Eating and dietary restrictions are often a part of the lives of children and adults with intestinal failure. Eating was not addressed as a separate domain in the surveys and was only present on three proxy rated multi-item scales: one item on both the ITQOL physical abilities and growth and development scales and one item on the 28-item Functional Status IIR. The questions related to the parents satisfaction with the child’s eating rather than the impact of having to restrict dietary intake and utilize specialized formulas. This topic was explored in the focus group, where a father reported the difficulty of having to explain to a young child the reason for their food and fluid restrictions. The mothers of infants in the study by Gottrand et al. scored their children lower in terms of eating compared to published norms\(^63\). Another aspect of intake that presented a struggle for some parents in the focus group was the presence of an oral feeding aversion. Engstrom et al found that the three children (aged 8-10 years) did not have an issue with hunger but the mothers complained of having to deal with a refusal to eat\(^67\). The children complained in that study of being pressured to eat by parents and healthcare providers. This aspect was not mentioned in our focus group however should be explored in future studies.

The ITQOL proxy questionnaire addressed the satisfaction of the parent with the child’s growth and development, but was not assessed in the other generic questionnaires. The parents of children less than 5 years of age on home PN scored their children significantly lower than the normal population on this variable. Gottrand et al. also found that parents were not satisfied with the infant’s development in terms of eating and speaking\(^63\). This was not discussed in the focus groups however parents did express their concern with the impact of long-term hospitalization on development. The absence of its mention in the focus groups may be a lack of data saturation or in the context of readjusted expectations. An abstract by Ament et al. from UCLA reviewed 8 children between the ages of 4 and 8 years old who were receiving 75% of their calorie
requirements since infancy. They utilized multiple tests and found that the children functioned in a normal or borderline range of intellect with strength in verbal conceptual skills and weaker results in visual-spatial planning ability. Their overall conclusion was that home PN allowed for normal cognitive development. Children with chronic illness often do not develop at the normal rate of healthy children. Stein discussed how difficult it is to determine the reasons for a child not reaching their developmental potential and stated that it may include an abnormal developmental process, the environment promoting dependence (hospital or home) or truly due to the underlying disease. Canam postulated that if the parents focused too much on the illness or disability, that they may exclude their developmental needs and as a result limit their child’s development. In this study, the parents of children less than 5 years of age on home PN were not satisfied with their children’s growth and development which included assessment of motor skills, physical changes, language and cognitive development, eating, sleeping, bowel habits, general temperament and overall growth and development.

Upon review of the weight and height z-scores it was found that some of the children were within normal range but others were greater than 2 standard deviations below normal indicating severe underweight and stunting. This may be very stressful for children who want to feel and look like their peers, as well as for their parents. This question was not addressed in the quantitative surveys. When Silver interviewed the 3 children on home PN between the ages of 8 and 10, they expressed that the only reason they felt different from their peers was because of their difference in growth and all stated they were the shortest in their class. This aspect should be investigated further in the older children on home PN.

Finally, the home PN patients’ functional status was examined by proxy using the Functional Status IIR and was found to be significantly different than the healthy reference population. This indicated a lower overall functional status relating to their chronic illness. Functional status was one aspect of the multidimensional QOL construct in this study, as functional status on its own is not equivalent to the lived experience.

6.4.4 EMOTIONAL AND SOCIAL DOMAINS

The children that were rated by proxy on the ITQOL (less than 5 years of age) were not rated significantly different in terms of behaviour, temperament and moods and getting along with
others. In contrast, the children that were greater than 5 years of age were rated significantly lower in terms of role and social limitations owing to emotions and to behaviour on the CHQ-PF50 and the emotional and social functioning scales and psychosocial summary score on the PedsQL™. These lower scores related to the perception that the children felt sad, angry, worried or had trouble sleeping, keeping up with peers, getting along with others, and participating in activities and school with friends. The parents perceived that these children had difficulty in keeping up in school and with friends due to multiple absences, fatigue and limitations in activities. The parents did rate the children similarly in terms of mental health, self esteem and behaviour.

The self esteem scale was not significantly different but had a very wide range of scores (from 8.3 to 95 of 100) suggesting a wide variation in opinion on what the children felt about school, athletic ability, friendships, family relationship, appearance and life overall. Engstrom et al. found that parents ranked their children as demanding a high level of attention, having lower social activities, less friends, rapidly changing emotions and believed that overall these children were frequently psychologically distressed. In our study, the focus group was primarily represented by parents of young children with only one child attending school. This parent reported that the child had difficulty making friends with children their own age and had an easier time engaging with adults. This was thought to be related to having spent a large part of her life in and out of hospital and developing relationships with adult health care professionals. Consistent with this finding, Gotttrand et al. reported that adolescents rated themselves significantly higher than healthy norms in terms of their relationships with adults.

There is potential for significant misbehaviour with chronically ill children if parents do not place the same limitations and boundaries for them as they do their other children. Canam emphasized in a review of adaptive tasks for parents with chronically ill children, that it was important to discipline their child and to place limits on their behaviour in order to develop their sense of security. However, difficulty with behaviour was not identified as a significant finding in the quantitative surveys nor was it mentioned during the qualitative aspect of the study. Engstrom used the Child Behaviour Checklist (CBCL) to assess behaviour in a Swedish home PN sample and found a significantly higher score in internalizing but not externalizing symptoms.
when compared to norms. It is possible that the children internalized symptoms more often than they externalized behaviours which would make it more difficult for parents to have recognized.

The child **self-report** (n=4) did not demonstrate any significant differences in relation to the CHQ-CF87 emotional and social scales: mental health, behaviour, self-esteem, role and social limitations owing to emotions and behaviour. There were, however, significant differences in the emotional and social functioning scales and the psychosocial summary scale on the PedsQL™ (n=6). These were consistent with the findings from the proxy assessments. The discrepancy in the CHQ-CF87 and PedsQL™ may have been a result of the small sample sizes and also due to the differences in item content on the compared scales. The scale for the emotional functioning scale on the PedsQL™ was similar to the CHQ-CF87 scale for mental health. However, while the emotional functioning scale focused on negative feelings such as fear, sadness, anger, difficulty sleeping and worry, the mental health scale included positive and negative feelings. It was possible that the children experienced a wide range of emotions, both positive and negative resulting in the higher scale score on the CHQ. The PedsQL™ social functioning scale indicated that these children experienced a more difficult time than published norms in terms of social relationships with peers. The scores on this scale in addition to the CHQ scales for self-esteem and behaviour (getting along with others) encompassed wide ranges demonstrating that these children had variable perceptions of their self esteem, relationships with others and social functioning.

There was significant variability in proxy and child perception of emotional and social functioning. However, there was agreement in the ratings that these children experienced more negative emotions and concerns than normal, healthy children and that they have more challenges in terms of their social experiences with peers.

### 6.4.5 SCHOOL FUNCTIONING

The PedsQL4.0™ had a specific school functioning scale which was scored significantly lower by **parent proxy** with an approximate 20 point mean difference (on a scale out of 100) in the home PN sample when compared to the healthy normative sample. This was consistent with Engstrom’s findings that parents ranked their children as having worse school performance. The **children** also scored themselves significantly lower in the school functioning scale.
compared to norms. This scale reflected the challenges of keeping up in school, paying attention in class and missing school due to illness or hospital appointments. There has been an improvement in survival for many chronic illnesses, including intestinal failure, outlining the importance of preparation and readiness for the school and schoolteachers. A study by Mukherjee et al. demonstrated that children with chronic illness had difficulty attending school regularly, meeting curriculum requirements and adjusting to the social demands of school\textsuperscript{174}. In the literature, many of the teachers have reported not feeling prepared for having these students in their classroom\textsuperscript{175}. The parents in the focus group described the process of meeting with teachers to educate them as well as the classmates. It was deemed by the families and recognized in the literature that increased knowledge may translate into an increase of support in both social and academic activities from the teachers and peers\textsuperscript{174}. One parent described how the transition to an alternative school was helpful for the integration of her daughter. Teaching was provided by and for the teacher and children in an age- and developmentally-appropriate manner to help them recognize her daughter’s challenges and was described as being highly beneficial.

Due to the challenges of periodic hospitalizations, appointments, and frequent illness, children on home PN had significantly more challenges in meeting scholastic expectations and attending school regularly. Educating the schoolteachers and peers about the child’s condition and challenges may assist with integration and support in the school system.

6.4.6 PARENTAL AND FAMILY IMPACT

Having a child with a chronic illness has a significant effect on the caregiver and the family. In addition, there is a reciprocal relationship between the child and the family so the impact that the child’s illness has on the family will influence the child as well. Thus it was essential to assess the overall impact on the families of the children on home PN. Family impact was measured quantitatively by the parental impact on time and emotion scales on the proxy completed CHQ-PF50 and ITQOL tools and the family cohesion scale on the CHQ-PF50, ITQOL, and self-report CHQ-CF87 questionnaires. In addition, a separate proxy report, PedsQL\textsuperscript{TM} Family Impact Module was completed.

The proxy completed Family Impact Module was developed to identify domains where a child’s illness or treatment specifically impacts the dynamic of the family or its ability to function. The
results from the home PN sample demonstrated low median scores (less than 65 out of 100) on almost all scales excluding the family relationships scale and the caregiver-specific cognitive functioning scales. The overall parent QOL summary score, the family functioning summary score and the total score results for this module were also very similar, scoring between 63 to 68 points out of 100. There were no published norms at the time of the study to compare the scales of the Family Impact Module, given that the tool is intended for parents of children with chronic illness. However, a study by Anthony et al. in a pediatric kidney transplant population from the same institution presented mean scores that were between 12 and 30 points higher on all domains and summary scores\textsuperscript{161}. The only domain that had a mean difference less than 10 points was the Family Relationships scale. These low scores demonstrating a negative effect on Family Impact were consistent with those of Holden et al. who found through the British Artificial Nutrition Survey (BANS) that families reported an overall worsening of QOL when their children were on home PN\textsuperscript{68}, as did Engstrom in a Swedish study\textsuperscript{3}.

The proxy ratings via ITQOL and CHQ-PF50 had a significantly lower score for the parental impact on time scale reflecting that the proxy respondent were limiting their own needs as a result of their child’s health, wellbeing, attention and/or learning. This was an expected finding as the parents of children on home PN had an enormous burden of care. The primary caregivers scored low on the Family Impact physical functioning score which reflected upon feelings of exhaustion such as fatigue, headaches, weakness and a sick stomach. This was consistent with a study by Sexton et al which demonstrated that parents felt physical exhaustion\textsuperscript{71}. Margaret Rhone also found that the parents with children on enteral and/or parenteral nutrition experienced physical exhaustion and felt time-pressured\textsuperscript{176}. Although, physical exhaustion was not specifically mentioned in the qualitative component of the study, one father did articulate that “sleep is a myth” with emphatic agreement from the other participants. Sleep deprivation was a theme that has been prevalent in the caregiving literature\textsuperscript{74}. This theme was also present in home PN studies by Sexton, Holden and Wong which they believed to be a significant contributor to physical exhaustion\textsuperscript{68,69,71}. Monitoring for caregiver burnout is essential as this may result in poor adherence and technique in PN procedures\textsuperscript{177}.

The significantly lower score on the parental impact on time scale also indicates that the parents believed that they did not have time for other activities other than caring for their chronically ill
child, consistent with the theme of time-pressure in the study by Rhone et al\textsuperscript{176}. Results from the focus group described that caring for a child with intestinal failure on home PN resulted in a lot of physical responsibility in addition to the administration of PN including frequent changing of diapers or ostomy appliances, preparation and administration of enteral feeds, repeated changes of bedding and clothing due to enuresis, all of which were also described in the study by Sexton et al\textsuperscript{71}. Additionally, young children can be especially challenging as they tend to touch, bite, pull and pick at their central line so it was essential that parents ensured the line was secure and concealed under their clothing\textsuperscript{178}.

Wong et al. employed a questionnaire and reported that parents with children on home PN experienced worsened social, sexual and work lives\textsuperscript{69}. Parents in the focus group expressed that they had minimal time for additional social activities because of the responsibilities of caring for a child on home PN and also due to self-imposed isolation. This isolation was a result of not trusting others to care for the child on home PN and not utilizing respite care services. A Swedish study described consistent results with only 25\% of families stating that they could rely on someone else to do the hook up or disconnection of the PN. In the focus group sample, only 1 of 5 families utilized regular respite and nursing care to assist with the administration of the PN and home responsibilities. The challenge of learning to trust others to care for a chronically ill child and to allow others to perform tasks that have always been your own is widespread across illnesses\textsuperscript{179}. However, Canam and Coyne found that parents who reorganized responsibilities, formed a stronger support system, and allowed others to share the burden of care had a greater ability to demonstrate improved adaptation and coping\textsuperscript{173,180}. In addition, some form of respite care was important to prevent caregiver burnout and fatigue\textsuperscript{177}. Social activities outside of the home were difficult to participate in due to the schedule of the child on home PN and the responsibility of caring for a medically complex child. There was also evidence of social isolation of caregivers in a study on technology-dependent children\textsuperscript{74}. The impact on sexual life was not discussed during the focus groups although conversation ensued regarding the impact on marriage from having a child on home PN. The parents reported being separated for extended periods during long-term hospitalizations, having the young children on home PN sleeping in their rooms at night for fear of the central line being pulled out and also the lack of additional caregivers to facilitate having time together as a couple. Wong et al. interviewed parents of
children on home PN who felt they did not have the time to spend with their partners. Marital stress was reported more commonly in the adult home PN literature and was not described as frequently in the pediatric PN literature. It was difficult to determine whether there truly was not an increase in marital stress or whether this sensitive topic was not discussed because of the lack of privacy and discomfort in a public, focus group discussion.

Certainly having a child on home PN had also influenced the work lives of the parents due to many missed days with appointments, hospitalizations and children feeling unwell. Eight of 16 families in the home PN sample had single parent incomes in order to allow the mother to provide full time care to the child. In the focus group, it was expressed that this had the potential to impact the identity of the caregiver and also created financial stress. As one mother described, her husband was seen as a person when he went to work, whereas being at home robbed you of your identity and was very lonely at times. Vanneste et al. reported a negative impact on anxiety in mothers who were unable to have a job outside of the home which was consistent with the discussion in the focus group related to perseverating on medical issues that arose with their sick child. The study by Gottrand et al. where most primary caregivers were mothers, demonstrated that they rated their QOL lower than that of the moms of healthy children. Upon comparison with the father of the home PN children, the moms scored lower on work, inner life and freedom. A study by Tarbell et al. demonstrated higher parenting stress in fathers compared to mothers of children post liver and/or small bowel transplant. These fathers were very involved in the care of the children and thus the authors queried whether the amount of stress was directly related to the caregiving responsibilities of the parent.

The ITQOL and CHQ-PF50 proxy ratings scored significantly lower for the parental impact on emotion scale reflecting worry and concern related to their child’s health, wellbeing, attention and/or learning. This was consistent with the result from the Family Impact Module worry scale which was one of the lowest median scores on the scale (45 out of 100), indicating a high level of worry. This scale was related to worry about medical treatments, side effects, impact of the illness on other family members, reactions from others and their child’s future. A pilot study of the anxiety level of mother of 10 home PN children in France demonstrated that their anxiety levels were highest at diagnosis, decreased once they were trained in home PN and lowest at follow-up unless the child had a poor prognosis. However, the side effects of PN can be
significant and include thrombosis, repeated septic events leading to loss of vascular access as well as IFALD. Both the loss of vascular access and IFALD are indications for small bowel transplant. Certainly this fear of complications was discussed during the focus groups. Many parents expressed concern about the risks of infections, sepsis, and loss of vascular access however only one mother mentioned IFALD. This was an indication of the new generation of home PN families that are followed at this institution. The prevalence of IFALD was much higher prior to the initiation of a multi-disciplinary team, modifications in PN constituents and the inclusion of Omegaven®, an omega-3 lipid emulsion, as rescue therapy for children with a conjugated bilirubin approaching 100 umol/L. The concern of IFALD was much more widespread 5 to 7 years ago as was the fear of being listed for a SBTx. In fact, there was not a single mention of transplant during the focus groups by these parents. The study by Silver et al. 6 years ago demonstrated that all 3 mothers and children expressed fears regarding the complications of being on home PN with the greatest fear relating to the development of IFALD. This was also documented in open interviews of 48 adult patients on home PN. Other parents had been documented to generally fear for the future of their children on home PN. This was not directly mentioned in the focus group however, upon review of the surveys it was noted that many parents were uncertain about their child’s future although did not necessarily assume the future would be negative. There was an element of hope expressed during the focus group that the children would have their PN discontinued one day and that they would be able to sustain their nutritional and metabolic status without intravenous support.

Another aspect of worry on the Family Impact Module was the impact of having a child on home PN on the other children in the family, which was also reported in the study by Rhone et al and discussed at length in the focus groups. The parents expressed the need to isolate the other children at times in order to perform sterile central line care, PN administration and cessation. Also, there were many activities that they were unable to participate in as a family because of the child’s central line, risk of infection and PN scheduling. This included going to beaches, camping, water parks, and most events that took place in the evening. Gottrand et al. included siblings in their QOL study in France, most were self-response and a small number were assessed by parent proxy. They found a similar QOL in the siblings as for the children on home PN, which was comparative to published norms. However, the siblings had a higher
satisfaction with eating and health\textsuperscript{63}. It can be challenging to maintain positive relationships with the siblings of chronically ill children as the ill child is often the centre of attention. It was stated very clearly during the focus groups that these families plan their lives around the child on home PN. Canam discussed the importance of balancing the needs of the ill child with that of the whole family since continually focusing on one child is often at the expense of others and can negatively impact their self-esteem\textsuperscript{173}. A meta-analysis on siblings of children with chronic illness was conducted by Sharpe et al. and found that in comparison to normative populations, there was a greater negative effect on these children in terms of internalizing behaviours such as anxiety and depression\textsuperscript{182}. There appeared to be less of an impact on externalizing behaviours, which may explain why parents in the focus group felt that their other children never complained and were adaptive to having a chronically ill sibling. In addition, the meta-analysis demonstrated that the negative effect on siblings of children with chronic illnesses was related to the frequency and amount of parental attention and caregiving demands required for the ill child\textsuperscript{182}. This would suggest that siblings of children on home PN could be greatly impacted since PN infusions and central line care occurred, for most of these children, on a daily basis.

The family cohesion scale is a single factor item asking the proxy and child to rate their family’s ability to get along with each other. This scale demonstrated non-significance in both the CHQ (self and proxy) and ITQOL surveys when compared to the normative values. This was consistent with the result of the family relationships scale on the Family Impact Module which was scored 72 out of 100. The parents confirmed this aspect in the focus groups discussing the fact that they had all committed to dealing with their child’s illness and treatment as a team and that they were in this together\textsuperscript{1}. The parents described spending a lot of time together as a family and one mother expressed that her other children appreciated the value of family more than other children their age. These were in contrast to literature that reported deterioration in family relationships for those families with children on home PN\textsuperscript{3,68,69}. In a 2002 review of families and chronic illness, it was noted that there was a discrepancy in the literature and that some evidence proved families continue to function well and that others showed negative outcomes for family functioning\textsuperscript{183}. Family cohesion has the potential to be a time-dependent factor as well. There is a possibility that initially the family bonds together to cope with the challenges and that over time this cohesion weakens. This aspect would benefit from being studied longitudinally.
The family activities score on the CHQ surveys was significantly lower than the published norms and related to the impact of the chronically ill child on spontaneity, cancellation of plans, limiting activities and participation in everyday activities. The daily activities scale on the Family Impact Module was one of two of the lowest median scores (approximately 40 out of 100) exemplifying challenges in having energy and time available for household tasks and the impact of the child’s health on the time taken for family activities. All of these results were confirmed in the qualitative aspect of the study. It was expressed in the focus groups that due to having a child on home PN with an unpredictable clinical condition, it was difficult to be spontaneous or even to plan activities without having to cancel them. The parents expressed that their central focus needed to be on the ill child and activities were planned around the infusion schedule which ranged in this study from 10 to 18 hours daily. Furthermore, they were tied to delivery schedules of supplies and PN solutions. They also discussed that everyday activities could be complicated: “Even a bike ride is complicated”. This aspect was confirmed in other studies demonstrating that families with children on home PN had lower social activities and was also present in studies of adults on home PN.

Parents of children on home PN had a low overall QOL as scored on the Family Impact Module. The medical responsibilities related to caring for a child on home PN significantly impacted time, limited social activities and limited attention to the personal needs of the caregiver. The caregiver experienced negative effects related to social isolation, inability to work outside the home and balancing the dual role of parent and nurse. There was a pervasive impact on the entire family affecting both the marital relationship as well as the lives of the siblings by limiting family activities and spontaneity of plans. The siblings may be at risk of internalizing behavioural symptoms, such as anxiety and depression, as a result of living with a chronically ill child with high caregiving needs. Despite the negative impact on family, family cohesion was found to be very good in this study.

6.5 The Lived Experience of Parents of Children on Home PN

Parenting a child with intestinal failure on home PN is a challenging and life-altering experience for both the parents and the child. Due to many uncertainties and responsibilities, life becomes a time of continuous stressors and periodic crises, common to many families of children with
chronic illness. Historically, fewer children went home on PN and more patients developed IFALD and died before receiving a transplant. In all chronic diseases that have an increased rate of survival but no immediate cure, the parents go home with their children and a heightened sense of uncertainty.

Quality of life was expressed by these families in terms of happiness, giving the child the best they can, an opportunity for socializing, the achievement of normalcy and the recognition that their QOL was relative and dynamic. With the discussion of normalcy, there was recognition that normalcy was relative and that all of these parents had to adapt and reassess their concept of normal. “And we create our own sort of normality, basically, in our household. This is what is normal to us now.” A literature review of needs of parents with chronically ill children also found that parents had to make significant lifestyle changes and accepted these changes as a new normality.

The focus group results demonstrated that these parents had encountered many different experiences throughout the course of their child’s illness. They had to let go of or re-evaluate expectations associated with activities they had imagined they would do with their child when they were presumed to be healthy and have since learned to live one day at a time. Eakes et al. found that in learning to cope, many families of children with chronic illness took on a ‘can do’ and ‘one day at a time’ attitude in order to help manage the condition. In addition, they tended to concentrate on better aspects of family life. The parents in the focus group discussed this concept and expressed that they truly appreciated time with their family and the small things in life. They felt that families with healthy children take common events such as bike rides and picnics for granted. Whereas families with children on home PN appreciated that a time when their child felt well enough to participate in an everyday activity, the other children were happy and the parents felt good, was an amazing thing to be grateful for. In the review by Knafl et al., they described that families found positive meaning in experience initially and incorporated illness management into everyday routine and at some point the family life took back on a taken-for-granted quality. This concept was not observed in the focus group of families with children on home PN however overall the children were still quite young. The parents were generally positive overall during the focus group and expressed how grateful they were that the PN therapy was available for their children. They were aware that had their child been born
greater than 30 years ago, they may not be alive. One mother said "You know, look at what you have instead of what you don’t have”. This emphasis of favourable outcomes has been proposed to assist with parents coping and to maintain a sense of hope. Despite the positive attitudes and alluded acceptance of the illness, there was also emphasis that none of this was a choice: the diagnosis, the central lines, and the home PN therapy. However, the reality was stated that it was the support line for their children. Comparable results were reported in a study that interviewed 3 mothers and found they appreciated that PN therapy was their child’s chance for life, recognized that there were improvements in their ability to function however expressed that there were still multiple physical and psychological challenges associated with this treatment.

Taking a child home on PN therapy requires accomplishing time consuming and medically complex therapies incorporated with the regular responsibilities of parenting. Parents discussed the shift of family roles and one family provided examples where siblings were assisting in the care of the child on home PN. This effect has also been documented in families with children who have childhood cancer. The parents discussed the stress that accompanies the burden of caring for a child with a medically precarious illness. Ray et al. described how parents need to acquire clinical assessment and decision-making skills when they bring a child home with a chronic illness. Parents with children who are technology-dependent are required to learn about illnesses, symptoms, medications, technological treatments, and to monitor for life-threatening side effects. As a result of the newly acquired responsibilities, the home PN parents have taken on a dual role of nurse and parent, also seen in a study of parents of technology-dependent and medically fragile children. Parents described the stress of having to monitor for life-threatening complications such as infection and thrombosis, as well as, needing to be highly diligent in administering PN and conducting sterile procedures. Caregivers in the studies by Silver and Sexton et al. also described the fear of coming home initially on PN and the burden of care that was associated with having the children at home. In order to achieve some control over the situation, some parents expressed that they had a system in place for delivery and ordering of supplies, as well as, the medical care of the children. As per Knafl et al., mastery and routine of the treatment are important in the family’s response to illness.
In addition to monitoring, the families had to learn to assess their child’s clinical presentation and the severity of their symptoms to determine whether to call the responsible medical team or to present to the emergency department. As one parent described it “You have to be on your toes all the time”. Presentation with common symptoms such as a fever, stuffy nose or cough often resulted in a significant medical work up including bloodwork, x-rays, etc. This was felt to truly impact their child’s QOL because if it were a healthy child, there would be a minimal medical work-up in comparison.

There was a constant struggle with their desire to allow these children to engage in activities and to truly experience life with the potential risks of infections and damage to the central line. This theme was recognized in a book on the effect of chronic illness on children and their families. There was a need for parents to weigh the potential risks to the child’s physical health against the benefits to the emotional health in allowing the child to engage in activities\(^ {184}\). Silver found that families described finding new interests and recreational activities to help adjust to their new lifestyle in both children and adults on home PN\(^ {67}\).

Intestinal failure is not a common diagnosis and there was an element of reported isolation in having a child with a rare disease. Parents found themselves confronted with health care professionals who were not familiar with the disease or its treatment. The parents expressed that this resulted in many stressful visits to emergency and to other hospitals, as a result of being on vacation or living away from the primary medical team. Parents became the experts on their children from having the responsibility of continually caring for their chronically ill. It can cause significant tension when medical teams do not treat families as part of the team. There is evidence that family\(^ 6\) who had a good relationship with their health care team managed illness more effectively\(^ {190}\). A study on the level of mother\(^ 7\) anxiety in home PN showed positive influence on confidence and anxiety with continued care and close follow-up\(^ {70}\). Overall, the parents in the focus group reported having an excellent relationship with the intestinal failure team and felt that the health care professionals had a true understanding of the needs of their child from a physical and emotional standpoint. There is a gap in the literature looking at the impact of health care professionals in shaping the response and coping of families to chronic illness\(^ {183}\). However, given the amount of discussion in the focus groups that was related to the primary medical team, it was evident that there was a significant although undefined impact.
The families expressed concern in terms of outpatient communication and described the challenges of adapting to this process in comparison to the communication received as an inpatient. As an outpatient, medical teams triage patients according to medical urgency. Therefore, it may take time to receive a response if the concern is not viewed as urgent. It was described that being the parent at home with the stress of monitoring your child conveyed a sense of urgency in their minds. Thus, the process of not receiving an immediate response was deemed to be very stressful. They described a natural disconnect of the level of concern when a team had multiple children they were responsible for and the parent had just one, especially given that monitoring the child is an essential aspect of their jobs as caregivers. Another topic of concern that was discussed involved the process of having to go to emergency when their children were unwell. The emergency department, despite the precautions taken to prevent the spread of infectious diseases, was seen by these parents as a place that their child would get sick. “If she’s not sick already, she will be by the time we leave [...]”. This perception created an enormous amount of stress for these families. In addition, with the exception of one parent, the other parents shared the belief that the physicians in the emergency department were unfamiliar with their children and most aspects of their care and therapy. The parents felt that the act of repeating their child’s very complex medical history was especially frustrating. The intestinal failure team had recently written letters of history for these families to bring to emergency in order to reduce the stress in response to this concern; however this was not mentioned during the focus groups.

In order to achieve a sense of normality, the families reported trying to engage in activities that other families with healthy children participated in such as going to the park, the zoo, having play-dates and family outings, etc. The one aspect that they believed was very important but highly difficult to engage in was to take a family vacation. One family with an older child had significant experience going on vacations, although her daughter had been on and off PN over time, but most of the families felt that the task was daunting. This was consistent with the findings of parents interviewed in an unpublished study by Wong et al. who expressed difficulty in being able to take holidays. A survey of 347 adults on home PN also revealed their frustration with the challenges of going on vacation however, the longer the duration of home PN, the more these adults adapted and took vacations. In order to take vacation, the families
were required to organize the supplies and the solution, secure appropriate storage space for these items for travel, ensure a location to perform sterile procedures and upon arrival, locate a nursing station or hospital nearby in case of unforeseen circumstances. One mother expressed that it wasn’t worth it for her and her husband while another felt that it was too stressful to be far away from the primary medical team who she believed understood her daughter’s condition the best. Vacation was thought to be extremely complicated to organize but also expressed to be important to all of those involved in the focus group, as it was opportunity to be a family and to participate in a normal family event.

As a result of the Ontario Health Insurance Program (OHIP), the PN supplies and solutions were medically covered for the families on home PN. The families in the focus group expressed sincere gratitude for this. However, they did outline that there were still numerous aspects of home PN and intestinal failure that could be financially challenging. Some families had to have their homes reorganized in order to accommodate the large number of supplies, PN and intravenous solutions. One family had to have their electrical rewired to accommodate 5 intravenous pumps running simultaneously. With numerous medical appointments, the families are required to spend money on parking in a large downtown hospital and despite family and parent parking rates, the price is considerable and adds up over time. The parents also reported costs associated with running a laundry machine and dryer multiple times daily as a result of stool or urine contamination of the bedding and clothing. There was a reported cost associated with replacing bedding due to frequent washing and wearing out of materials. Although there were cost analyses of PN in the literature, including comparisons between home and inpatient PN provision, there were no formal assessments that were related to the hidden costs of PN.

The parents expressed that the causes of intestinal failure and the need for home PN were numerous many of which were not diseases that, as they stated, ‘fit in a checkbox’. They alleged that there were many grants and funds available for ‘checkbox diseases’ e.g. cardiac, oncology, renal, and transplant, but that unfortunately their children did not meet the criteria. As a result, the resources available for these families were limited. There were a small number of families that lived out of province and were co-managed by their local team and the intestinal failure team. One family wrote a long note on their questionnaire explaining the stress related to being out of province. They stated that their local team was unfamiliar with home PN and found they
had very little support in obtaining resources. The benefits received in Ontario for home PN were not available in every province. This family expressed significant stress being one of very few patients on home PN in their town and stated that they had to figure it out on their own. Despite this, the drive to ensure that their child was at home and believing that their child and their own QOL would be significantly better in the home environment, they persevered. This mother, however, felt that her QOL would have been much better had the appropriate support been instituted.

These families explained that no matter what the challenges were, the best part of the therapy is that it was able to be administered at home. This allowed the family to be together and for the child to live out of hospital. They felt that the QOL they were able to achieve out of hospital would not have been possible had their child been required to stay in hospital for PN therapy. This sentiment was also relayed by the parents interviewed by Sexton et al.71

Support for parents, the families, the children and their siblings is essential. Meleski discussed the concept of family-centred care in an article related to helping families with chronically ill children learn to cope. The components that were identified as essential included collaboration between parent and provider, communication concerning the child’s needs, recognition of family strengths, acknowledgement of the family’s developmental needs, a responsive health care delivery system and a comprehensive support for the family including a parent-to-parent support group179. The parents in the home PN focus group described having access to resources for the chronically ill child however not for the parents themselves nor the siblings. The parents felt that this was inappropriate in an institution that promoted family-centred care. This was also described by Engstrom et al. where mothers expressed how stressful it was to find their own source of support3. The parents in our study expressed that there was a difference in having health care providers discuss the home PN experience versus hearing this from other parents that had actually experienced this with their own children. There was very positive feedback in terms of the focus group because for many, this was their first opportunity to engage with other parents who were going through similar challenges. They found it very therapeutic and were hopeful that something similar could be organized either face-to-face or online for parents who did not live locally. In addition, it would be beneficial for both siblings and children on home PN to
have access to peer support groups in conjunction with support provided by health care professionals.

The focus groups identified many themes that were not captured by the quantitative surveys and allowed for a better understanding of the lived experiences of having a child on home PN. Additional focus groups involving more parents and the children themselves would help to further enrich this body of knowledge.

6.6 Comparison to Published Small Bowel Transplant Populations

The QOL in the home PN sample was also compared to published data of children who had a SBTx at two different locations in the United States: Nebraska by Sudan et al. and UCLA by Ngo et al.\textsuperscript{18, 83}. Children on home PN who lose vascular access or develop IFALD are listed for SBTx. As outcomes for SBTx improve, SBTx may eventually be considered an alternative to long-term PN, thus it is essential to assess the differences in QOL between the two groups.

There were no published studies that compared Home PN to SBTx in pediatric patients. The QOL data published for the SBTx samples utilized the proxy reports only in the UCLA sample (CHQ-PF50 and PedsQL\textsuperscript{TM}) and both child & proxy reports in the Nebraska sample (CHQ-PF50, CHQ-CF87, PedsQL\textsuperscript{TM} proxy). There was no published data for SBTx patients using the ITQOL, preventing comparison of children less than 2 years of age.

6.6.1 OVERALL QUALITY OF LIFE

Small bowel transplantation and life after transplantation in general are still considered a state of chronic illness. When children receive a SBTx, the PN is typically discontinued however they may still require intravenous fluid to manage high output ostomy losses. They are required to go to hospital for regular follow-up including clinic appointments, frequent bloodwork, biopsies to monitor for rejection, and medical procedures to assess side effects. They take immunosuppressive medication for life that can cause long-term adverse effects and often require frequent hospital admissions for the first 1 to 3 years post transplant, as well as life-long surveillance and follow-up. There was a study that demonstrated that burden of care of SBTx
does decrease over time providing the child survives the transplant and does not develop significant morbidity\textsuperscript{21}.

In terms of the quantitative comparison using the PedsQL\textsuperscript{TM} proxy report, there was no significant difference in the total QOL score in the home PN sample compared to the UCLA SBTx sample. In the original abstract, Ngo et al. did not provide a comparison of their total score to published norms however it was likely that their scores were significantly lower than published norms as they were similar to the results in our home PN sample\textsuperscript{83}. In the adult literature, there were conflicting reports as to whether the QOL in patients on home PN was the same or worse than those who had a SBTx. Dimartini et al. found that patients who had a SBTx reported a significant improvement in many areas of QOL and no worsening at all\textsuperscript{87}. Both the home PN and the SBTx groups retrospectively reported a worsening of QOL from life before starting PN to after starting PN\textsuperscript{87}. Cameron et al. queried whether QOL was related to the stability on home PN. This study evaluated the differences of QOL between stable home PN patients versus unstable home PN patients (complicated intestinal failure assessed for SBTx) versus post SBTx patients\textsuperscript{91}. They found a similar QOL in the SBTx population and the stable home PN population and found that their QOL assessments were both significantly better than the unstable home PN patients who were considered ‘complicated intestinal failure’\textsuperscript{84}. In our study, there were no children in the home PN sample that would have been classified as unstable home PN. There were no differences in overall QOL in patients on home PN with the published SBTx sample as measured by parent proxy.

6.6.2 GENERAL HEALTH DOMAINS

The global health item and general health perceptions scale on the CHQ-PF 50 proxy report were not significantly different between the home PN sample and the two groups of SBTx patients. There was no published normal comparison for the global health item. However, in relation to the general health scale, the proxies of both the home PN and SBTx populations scored less than 50 (out of 100) resulting in significant differences compared to normative populations. In contrast to the proxy results, the children on home PN scored themselves significantly lower on the general health scale and the global health item in comparison to the SBTx ratings. This suggested that the children on home PN felt that their general health was
worse than the patients who had a SBTx and that they likely had more concerns about their past, present and future health status. Although children post-transplant require daily medication, the children on home PN were connected to intravenous therapy nightly that impacted sleep and nocturnal enuresis. That may have been a more powerful reminder that they did not share the same health status as children their age. The children post-SBTx, unlike the parent proxies, assessed their general health as similar to healthy norms. It was possible that parent proxies had a better understanding and recollection of the time when their child was unwell pre-transplant. The SBTx parents may have also had a better appreciation of the potential side effects of immunosuppression and thus experienced greater concern for their child’s future health.

Overall, the parents of both the SBTx and the home PN samples did not feel that their children’s general health was dissimilar however they both felt it was poor compared to healthy children. The children in the home PN sample had a perception that their general health was worse than those in the SBTx group.

6.6.3 PHYSICAL DOMAINS

There were no significant differences in the proxy report physical functioning scale of the PF-50 when the results from the home PN sample was compared to the Nebraska SBTx sample nor in the PedsQL™ physical functioning scale for the UCLA SBTx sample. In the original publications, both of these groups were significantly different than the normative population which is consistent with the results in this study for the home PN sample\textsuperscript{18,83}. The children who provided self reports using the CHQ-CF87 from Nebraska reported non-significant physical functioning scores in comparison to the home PN population. Consistent with the results from this study, there were also no significant differences reported when compared to the normative population in the original study. A study by Wake et al. that surveyed adolescents with diabetes found similar results and hypothesized that this may have been related to teens not wanting to be perceived differently and answering as they felt they should\textsuperscript{125}. Although this was also a possibility in this study, there was no data to support this in any of the studies. This could be investigated further in a qualitative assessment of these children on home PN.

Bodily pain as assessed by the CHQ-PF50 proxy report scale was lower in the home PN sample by approximately 10-15 points compared to the two SBTx groups, however there were no
significant differences. It was difficult to assess whether a true difference existed and was not detected due to the small sample size or if there was a clinical difference. It was noted that the variability in the home PN sample was large with a wide range of responses and therefore a larger sample size may have provided a better indication of the true mean. In comparison to the child self-reports using the CHQ-CF87, there were no significant differences with bodily pain. From a patient perspective, bodily pain does not appear to differ between the two groups. In the proxy assessment, it appeared that the caregivers of the home PN children had a wide variability in their opinion on their child’s experienced pain. In 2006, an adult study by Pironi et al. used the SF-36 to compare QOL in 12 post SBTx patients and 18 home PN patients and found only one significant difference related to bodily pain which was worse in the home PN patients.

The results demonstrated that there was no difference in physical functioning between home PN and SBTx but according to proxy assessments, both function at a lower level than the normative population. In contrast, the children rated their physical functioning to be at the same physical level as their peers in both the home PN and SBTx groups. There were no significant differences in terms of experienced bodily pain by proxy and child assessment however, the proxy assessments demonstrated a wide variation in responses.

6.6.4 EMOTIONAL AND SOCIAL DOMAINS

There were no significant differences in the emotional and social domains between the home PN sample and the SBTx samples on any of the compared surveys. It was interesting to note that the scores in both the PedsQL™ and the CHQ-PF50 by the home PN and the SBTx proxies, demonstrated the same significant differences when they were compared to the normative sample. There were similar ratings in terms of role and social limitations related to emotions and both proxy groups rated the mental health and self esteem of the children comparable to norms. The only difference was with the behaviour domain where the UCLA SBTx sample scored the children significantly lower in comparison to norms but Nebraska SBTx and home PN did not. In addition there were no significant differences in the emotional and social domains between these groups using the PedsQL™. The proxies rated the home PN and SBTx children significantly different compared to norms in both the social and emotional domains demonstrating that both groups may have had more issues with negative emotions, such as
sadness, anger and worry as well as keeping up in their social lives with friends. There were no significant differences between the children in the SBTx and the home PN group using the CF-87 and both groups scored non-significantly different in the emotional and social domains against norms. In summary, there was very little difference in the ratings between the home PN and SBTx groups upon comparison, with similar results from both proxy and self ratings. The proxy ratings of the home PN and the SBTx groups rated the children significantly lower in terms of emotional and social functioning compared to normative populations. The home PN and SBTx self-ratings using the CHQ-CF87 resulted in non-significant differences in emotional and social domains compared to normative populations.

6.6.5 SCHOOL FUNCTIONING

The PedsQL™ school functioning scale was assessed by proxy for the home PN and SBTx group and no statistical differences were found. Both groups were rated significantly lower than normative samples which demonstrated that both the home PN and the SBTx proxies were concerned with their child’s ability to attend school regularly, keep up with the curriculum and to focus during school. The similar assessment between groups was not surprising as both populations have multiple appointments, frequent bloodwork, are at a higher infection risk, and may require periodic hospitalizations and medical procedures all which result in missed school days.

6.6.6 PARENTAL AND FAMILY IMPACT

In terms of parental and family impact there were no significant differences between the groups. There was a similar impact in the home PN and SBTx populations in terms of parental impact on emotion which revealed that parents worried about their child’s health and wellbeing more than parents of healthy children. There appeared to be a slight improvement in parental time for the SBTx group compared to the home PN sample (mean difference was 7 to 14 points higher in SBTx), although this finding was non-significant. Post transplant, these children were presumably off of intravenous infusions. Although the SBTx patients still required close monitoring and assessment for side effects and complications, there would be time available from not needing to perform sterile procedures and administer and prepare PN. A study by Hind et al. reviewed the impact of SBTx on parental mental health and found similar results. Based
on the results of 12 post-SBTx and 4 home PN parents, they determined that the scores from the General Health Questionnaire (GHQ) improved significantly for all scales except for the impact on parental emotion and time\textsuperscript{86}.

Both groups had lower scores than norms in terms of family activities but were not different from each other. Consistent with the findings from the CHQ questionnaires of the home PN sample, the SBTx sample had comparable scores to norms for family cohesion. The family cohesion item was about 10-15 points higher in the home PN group than the two SBTx ratings however this finding was non-significant. The Family Impact Module was not a part of the other studies thus could not be used for more detailed comparison of the effect on family. In terms of the CHQ-CF87 child ratings, there were no significant differences between the home PN and SBTx group and there was a non-significant trend of children on home PN scoring lower (17 points) on the family activities scale. This may have indicated that the children on home PN felt that their health had limited the types of activities that the family could partake in, scheduling and planning of family activities, interrupted plans and may have been a source of tension.

Generally, there were no significant differences on the family and parental impact scales between the home PN and SBTx samples. There were non-significant trends that may suggest a potential improvement post-SBTx in the parental impact on time as assessed by proxy and an improvement in the impact on daily family activities as assessed by the children.

### 6.7 Comparison to Institutional Small Bowel Transplant Patients

The results from the QOL battery assessment in the home PN sample was compared to the small sample of SBTx patients from the same institution. All of these children were survivors of intestinal transplant and had not required retransplantation, which was similar to the children in the study by Sudan et al\textsuperscript{18}. Of the 6 participants in the SBTx sample, 5 received a liver in conjunction with a bowel for the indication of IFALD. It is interesting to note that at the time of the study, there were no children from the home PN group listed for a SBTx and none with advanced liver disease, although one home PN patient had received a liver transplant greater than 3 years ago for that indication. This was demonstrative of the changing population of intestinal failure at this institution. There was a survival bias in the respondents from the SBTx sample.
due to the fact that at the time of the study only 7 of 14 SBTx patients were alive. In terms of the self-assessments using the PedsQL™, it was important to consider in the interpretation that approximately half of the results from the home PN group were based on adolescents whereas the results from the SBTx group were solely based on children less than 8 years of age. The results of the analysis of the home PN sample compared to the institutional SBTx sample were greatly limited by small sample sizes in both groups.

6.7.1 OVERALL QUALITY OF LIFE

The tools utilized for comparison of overall QOL between the home PN and SBTx groups were the VAS QOL scale and the PedsQL™ proxy and self-report total QOL scores. On the VAS proxy assessment, there was no significant difference between the two groups based on the assessment of the VAS result as a non-parametric continuous variable. However, when it was assessed as a categorical variable, there was a significant difference between the home PN group and the SBTx group. There were significantly more parents in the home PN sample who rated their child’s QOL as “low” (less than 8 cm) compared to the SBTx sample. There was only one child who rated themselves on the VAS scale in the SBTx group and they scored themselves 3 cm lower than the mean score in the home PN group however, the range of scores from the PN group was large. On the PedsQL™ there was no significant difference in the total QOL scores between the two groups on both the proxy and self-assessment. The PedsQL™ total QOL score proxy results were less than 10 mean points difference however the children from the SBTx group rated themselves 17 points lower than the home PN group. The difference in the age of respondents between these two groups, may attribute to this difference and be an indication that the adolescents on home PN had become more accustomed to living life as a chronically ill child.

There were no significant differences detected between the home PN and SBTx group in terms of the Functional Status IIR, both of which had a mean score of approximately 80 out of 100. This suggested that there were no perceived differences by proxy between the groups in terms of how their chronic illness impacted their daily functioning.

As measured by VAS, there was a difference in QOL with the home PN population having a lower QOL than the children post SBTx from our institution. However, in terms of the results of the multi-item questionnaires, there were no differences between these two groups which is
consistent with the comparison to the published SBTx population. In addition, functional status as an aspect of the multidimensional construct of QOL was not significantly different between the two comparison groups.

6.7.2 GENERAL HEALTH DOMAINS

There were no significant differences between the two groups for the general health or global health scales on the proxy assessed ITQOL and CHQ-PF50. However, on both tools the home PN and the SBTx proxy groups had very low mean scores (30-38 out of 100) with similar standard deviations on the general health scale. This indicated that proxies from both groups felt concerned for their child’s health and were uncertain about their health in the future. The global health item asked what parents thought their child’s health was like in general and was also scored similarly between the two groups. Although, the parents completing the ITQOL for children less than 5 years of age, scored a mean of 8 points lower in the home PN compared to the SBTx suggesting that they may have perceived their child’s health to be worse. There were more babies and toddlers in the home PN sample which may have contributed to this difference. These parents would not have had as much time to adjust to the child’s chronic illness and may have perceived their child to be more unwell as a result. In addition, there were only 2 respondents for the SBTx ITQOL assessment. The global health item was scored more similarly by proxy for the older children via the CHQ-PF50. Overall, the median scores were low (<60 out of 100) demonstrating that both groups felt that their child’s health was good overall on a scale of poor to excellent. The VAS that measured the overall health was significantly different as a continuous variable with the home PN proxy assessment scoring the children lower than the SBTx proxy. There was also a significant difference when classified as high (≥8cm) and low (<8cm), with more SBTx proxies rating their child’s overall health as high. The VAS for self assessment was unable to be compared as only one child completed for the SBTx sample however it was interesting to note that the range on the perception of overall health for the four home PN children was from 0.8 (worst overall health) to 9.7 (best overall health). This implied a wide variation in children’s perceptions and actual health states when on home PN.

Both groups completed the change in health score, which was not available for comparison in the normative and published SBTx populations. There were no parents in the SBTx sample that felt
their child’s health had gotten somewhat worse or much worse in the past year whereas a small number in the home PN population felt that was the truth for their child. The majority of the SBTx group felt their child’s health was much better than a year ago. Whereas in the home PN sample, the majority felt their child’s health was the same or somewhat better.

Overall, there was significant evidence to suggest that by proxy rating, the children on home PN had a lower overall assessment of health in comparison to the SBTx sample. This was in contrast to the comparison of home PN with the published SBTx data where the proxy ratings were in agreement. However, it was consistent with the self-ratings by the children on home PN when compared to published SBTx.

### 6.7.3 PHYSICAL DOMAINS

There were no significant differences on the physical domains in all administered QOL tools, including the ITQOL, CHQ-PF50, PedsQL™ self and proxy reports. The ITQOL questionnaire had similar median scores in the physical functioning and growth and development domains, whereas the PedsQL™ and CHQ-PF50 demonstrated non-significant, slightly variable median scores on physical functioning, in both home PN and SBTx patients. The self-ratings for the SBTx group showed lower non-significant median scores than the home PN group. However, these children were younger overall than the home PN group and may have had more difficulty with lifting heavy things, running, etc.

Bodily pain was not significantly different on the ITQOL nor the CHQ-PF50 proxy assessment. The mean difference in the bodily pain score on the ITQOL was 16 points lower for the home PN group however this was likely due to the wide range of responses. The median and IQR were essentially the same between the two groups. When overall pain was assessed on the VAS proxy scale, there was a significant difference as a continuous variable with the home PN group demonstrating an increase in experienced pain in comparison to the SBTx. When this result was categorized into high (≥ 8cm) and low (<8cm), there was no significant difference between the two groups. One mother made a note on the side of the scale stating that their child had discomfort but was uncertain as to whether it was pain. It was difficult to conclude that the patients experienced more pain as this would require a clinical interpretation of a 2cm VAS difference however; it was evident that there was a wider variation in parent perception of their
child’s pain in the home PN group and that there was a potentially higher sense of pain and/or discomfort in that sample.

In summary, the results from the comparison of the home PN to institutional SBTx patients demonstrated no significant differences in physical functioning. This was consistent with the proxy ratings of physical functioning when home PN was compared to the published SBTx literature. There was a significant difference in the proxy perception of their child’s bodily pain on the VAS scale, but not on the corresponding CHQ scale.

### 6.7.4 EMOTIONAL AND SOCIAL DOMAINS

There were no significant differences in the proxy and self assessments related to emotional and social domains between the home PN and the SBTx groups. The ITQOL temperament and moods scale was rated highly by both groups. There was a non-significant difference on the ITQOL getting along scale although this was difficult to interpret with only two respondents in the SBTx sample. The proxy respondent CHQ-PF50 demonstrated similar, high scores in self esteem, mental health, behaviour and the psychosocial summary scales. The emotional and social functioning scales on the PedsQL™ proxy assessment were also scored similarly. The PedsQL™ self assessment of the social functioning scale demonstrated a non-significant, large mean difference between the two groups, 33 points higher on home PN (out of 100). The home PN sample also scored 21 points higher on the psychosocial summary score. It was noted however there was a wide range of scores in both groups (10-95 for home PN and 0-70 for SBTx). These differences may have been related to age differences between the groups and the small sample size. However, it is possible that there was a more negative perception in the younger children (aged 5-7), post SBTx in terms of keeping up with other children their age, getting teased and making friends.

In general, there appeared to be no significant differences between these two samples in terms of emotional and social functioning by proxy or self-rating. This was analogous to the results of the comparison between the home PN and published SBTx data.
6.7.5 SCHOOL FUNCTIONING

There were no significant differences in the school functioning ratings on the PedsQL™ with proxy or self assessments. This was consistent with the proxy ratings comparing the home PN and published SBTx school functioning assessment. The parent proxy ratings for school functioning had very similar low mean scores (50-55 points out of 100) however, the median scores were higher with a small IQR range in the home PN group. In the self-assessment, there was a large mean difference in the scores for school functioning, 31 points higher for children on home PN and a lower score with a narrow range in SBTx. This result was surprising in that it was felt that children on home PN would have more difficulty in daily school functioning than SBTx due to more intensive daily therapy required although there is definitely a risk for both populations in missing schools days. In general, these results need to be interpreted with caution given the age difference of the children in the two groups. The home PN group had 3 of 6 respondents who were adolescents and the SBtx only had 3 children respond all of whom were between the ages of 5 and 9 years.

To summarize, there was no significant difference in school functioning from a proxy perspective between the home PN and SBTx groups. It was evident from the low overall scores that proxies in both groups had concerns with this aspect of their QOL. The child self-assessment results were more variable and difficult to interpret due to the age difference of the children.

6.7.6 PARENTAL AND FAMILY IMPACT

There were no significant differences in parent or family impact between the two groups as measured by items on the proxy respondent ITQOL, CHQ-PF50 and the Family Impact Module. The parental impact on time and emotion scales on both the ITQOL and PF50 were scored similarly by both groups although there was a wider range on the scales ranked by the parents of children less than 5 years on home PN. It was surprising that the parents on home PN did not have a lower score on the parental impact on time scale given the amount of work required for administration of home PN. Although non-significant, there was a difference in the median and mean scale scores for the caregiver social functioning scale. The home PN proxy scored lower
on this domain suggesting that they were more isolated, had less time and energy for social activities and may have had difficulty getting support from others.

There were no significant differences detected on the domains of the Family Impact Module. Most mean scores in both groups were relatively low (less than 65 out of 100) with the exception of cognitive functioning of the caregiver and the family relationships score. The parent QOL summary score, the family functioning summary score and the total Family Impact Module scores were within 5 points in both the mean and median scores between the two groups. In terms of parent QOL, although the SBTx families had experienced PN cessation there continued to be a burden of ongoing medical care which disrupted routine and contributed to anxiety about future health\textsuperscript{10}. These children continue to take daily medication, require diagnostic monitoring and repeated hospital visits, as well as have an increased risk for infection, rejection, graft loss and other morbidities associated with sustained immunosuppression. The protectiveness that was revealed in our focus group, is also present in other transplant literature in terms of opportunities and activities\textsuperscript{10}.

Both groups had the lowest scores in the worry scale and daily activities scale. This was interpreted as proxy concerns about their child’s treatments and side effects as well as the impact of their ill child’s condition on the rest of the family. One study of parents of children who were post liver and/or small bowel transplant demonstrated that parenting stress was high however was still within the normal range when compared to parents of children who were healthy\textsuperscript{85}. The daily activities scale was related to the time taken for family activities, being too tired or having minimal time to do household tasks. The family activities score in the CHQ-PF50 was scored quite low in the two groups as well with a slightly lower mean score and wider range in the home PN group.

There were no significant differences between these two groups in terms of family and parental impact. It appeared that the proxy respondents in both groups were impacted in terms of time, emotions, social activities, and experienced worry about their child’s treatment and future. There was some suggestion that the parents with children post SBTx may have had more time for social activities and less of an impact on parental time than those of children on home PN however, a larger sample size would be required to truly test these differences.
6.8 Limitations

There were a number of potential limitations with this study in both the quantitative and the qualitative aspects. These included a cross-sectional study design, a small sample size from a single centre, the use of multiple QOL instruments to reflect the wide age range of children studied, the use of parent proxy, the use of generic quantitative tools and the methodology related to the focus group.

6.8.1 CROSS-SECTIONAL STUDY

The study design was cross-sectional as intestinal failure is a rare diagnosis and a long-term study would not be feasible. Causality cannot be determined in cross-sectional design which limits the extrapolation of results. There were many potential factors that may have biased QOL at one point in time including recent changes in the child's clinical status and recent experiences of the proxy and child. Quality of life may have also been impacted by the home PN duration for that child. Chambers et al reviewed QOL in 27 adults and found an initial improvement in QOL over the first 6 months and then a flattening effect afterwards. In addition, QOL is a dynamic concept as mentioned by the parents in the focus group and thus a longitudinal assessment may have been more reflective of this changing concept.

6.8.2 SAMPLE SIZE AND SINGLE CENTRE

Sampling from a single centre limits generalizability and power, however the centre chosen was the largest and only formal intestinal failure program in Canada. The small sample size was related to the rare incidence of intestinal failure. This prevented the ability to do a multivariate analysis to predict medical and demographic factors that may have been contributing to QOL assessment e.g. the presence of stomas, gastrostomy tubes, duration on home PN. Harris's rule of thumb is that for five or less predictors, the number of participants should exceed the number of predictors by 50. This sample encompassed children of various home PN durations and stages of follow-up post transplant. Five of 6 SBTx children were transplanted for the indication of severe IFALD and at the time of the study, none of the children on home PN were classified as severe IFALD nor were they listed for SBTx. This demonstrated a changing demographic in
home PN which impacted comparisons, but also made it even more essential to describe the challenges and QOL that these families were facing. Engstrom et al. stated that it was difficult to assess QOL of children with intestinal failure on home PN due to the inability to differentiate between the impact of home PN versus the underlying disease\(^3\). This aspect was not addressed in the study however speaks to the variability of the sample. There was also a survival bias in the responding transplant population as 7 of 14 patients at this institution died after SBTx.

Although the 2 groups of children had similar mean ages, the SD was much larger for the home PN sample. The children in the SBTx sample were all between the ages of 2 ½ and 10 years whereas the home PN sample ranged from 3 months to 18 years. This limited the comparisons due to a lack of infant and adolescent survey results. This also had to be considered in the interpretation of available results.

6.8.3 CHOICE OF INSTRUMENTS

The choice of instruments for the battery assessment was aimed to reflect the multidimensional aspect of QOL as there was no comprehensive and ideal tool available to measure this construct. However, a different choice of instruments may have resulted in some alternative results. This was evident by the differences in some of the results of the two tools by Varni and Landgraf which were both intended to measure the same concept \(\text{QOL}\). However, it was noted that the initial goal of the CHQ tools by Landgraf was to measure functional status and the fact that the content of the comparable scales can differ may explain some of differences\(^9^7\). The population norms that were used for the CHQ-PF50, CHQ-CF87 and the PedsQL\(^\text{TM}\) were all American samples and the ITQOL was a Dutch sample. There may be significant differences between the norms and the home PN sample related to factors such as socioeconomic status, cultural impact due to region and country of residence, a different economic culture and standard of life.

In terms of statistical analysis, despite non-normal distribution, it was necessary to use parametric statistics without access to the original data of these published results for norms and SBTx. The effect sizes were used in terms of the statistical interpretation but were limited in that they do not necessarily translate into the actual impact in life\(^1^0^5\). In assessment of the VAS, the results were dichotomized according to a previous publication however, the use of different cut offs may have resulted in alternative outcomes.
6.8.4 PARENT PROXY

The majority of the children in the home PN sample were very young and thus the results were reliant on the use of parent proxy assessment. Parent proxy, with its limitations, is the only practical way to assess QOL in young children or in those children who are cognitively impaired, too ill or too fatigued to complete the instruments\textsuperscript{43,56}. There is variability in parents' ability to assess their child's self-concept and although the evaluations of parent and child may not necessarily be synonymous\textsuperscript{35}, the proxy/caregiver perception is arguably important. A child lives within a family structure and the coping and perception of their lives by the proxy or caregiver impacts their experiences and emotions. Varni et al believe that parent perspectives may be independently related to healthcare utilization, risk factors, and quality of care \textsuperscript{56}. Ideally, the parent proxy is utilized to provide multiple perspectives and a complete picture of the impact of disease in childhood QOL\textsuperscript{59}, however this was not possible with many young children in the sample.

Parent proxy and child agreement analysis was not completed in this study due to small sample sizes within the age-appropriate tools thus limiting power in both a correlational or agreement assessment. In general, there is thought to be more parent-child agreement in regards to physical versus emotional and social functioning however in the absence of a formal assessment, descriptively this was not consistent with the results of this study \textsuperscript{43,193}. In general, the children in this sample assessed their overall functioning to be better than the proxies however there was agreement in the assessment of their general health.

6.8.5 QUANTITATIVE SURVEYS

Quantitative surveys are limited as previously discussed in that a difference in chosen tools may result in a difference in outcomes. In addition, objective health measures do not necessarily translate into experienced QOL\textsuperscript{128}. A battery assessment was used to include aspects of the multidimensional construct of QOL, involving the use of generic tools only, as there were no disease-specific tools available. The generic tools risk asking too many potentially irrelevant questions and often miss disease specific issues. The focus group was used to offset this issue and it was expressed during the focus group that the parents felt that the generic questionnaires were not sensitive enough to identify many important aspects of having a child on home PN.
The measurement tools need to be appropriate for developmental stages and thus require several age specific versions. Having to use a multitude of tools to cross the age spectrum from 2 months to 18 years limits comparison of results during the analysis due to small sample sizes of age-specific tools. The majority of questionnaires were completed by proxy and specifically by the mothers. Some of the parents completed these together however it would have been interesting to have separate perspectives from both the mothers and fathers. In a study of parents post liver and/or small bowel transplant, the fathers actually presented with greater distress than the mothers. These fathers were very active in the care of the child. The questionnaires were completed both in the clinic setting and at home which may induce biases. In addition, many of the children completed the surveys at home and although it was discouraged, if they were helped by the parents than it may not be a true reflection of the child’s QOL.

6.8.6 BACKGROUND DATA COLLECTION

The background data was collected from a chart review with limitations of reliability and completeness of the data. Some data was not current or unavailable. The children from out of province are not followed with the same frequency and thus the details of the current medical and nutrition care plans were limited. There was a lack of sociofamilial data collected including the age and education level of parents, ethnic origin, and family income. As well, aspects of the child’s functioning such as classification of learning disabilities, use of special education etc. was not collected. This data would have contributed to the social information regarding the sample however with the limited sample size it would not have been used in the analysis.

6.8.7 QUALITATIVE FOCUS GROUP

The focus group was conducted in order to provide a more thorough and contextual understanding of the lived experience and QOL of children and families on home PN. The participants were chosen in order to maximize the duration of experience on home PN and the ages of the children. Overall, the age group was still quite young (< 8 years old), which also equated to reduced home PN durations and results may therefore not be representative of an older pediatric cohort. It was not possible to conduct a focus group with the children, themselves due to small number of children who were old enough to participate and their location of residence was often far from the hospital. It would have been useful to conduct more than one
focus group in the proxy sample in order to achieve data saturation. As per Morgan, it was difficult to know in a small sample whether the content of discussion was due to the unique characteristics of the participants or simply the group dynamics\(^9\). There is always a risk of participant bias in focus groups which is when participants behave in some way so that their responses are systematically different than they would have been in normal circumstances. The size of the focus group was appropriate as smaller groups are important for patients with a higher level of involvement in the subject matter\(^9\). One father and 5 mothers participated, however it would have been interesting to have additional fathers to gain multiple perspectives. Focus groups can be limited in extracting sensitive information for example, marital stresses and impact on sexual relationships etc. and thus individual interviews may have revealed other themes. Cross-sectional focus groups have similar limitations to the quantitative analyses in that families are influenced by recent events in their lives. Some discussion in the focus groups regarding recent occurrences confirmed this. Thus, if the focus group was conducted at different times, the data may have been different. The focus group also consisted of families that were proficient in English and thus the cultural variation was limited. It would be beneficial to conduct further focus groups or individual interviews with families with English as their second language to obtain a more thorough appreciation of home PN on various cultural groups. Sexton et al. found that Asian and Indian families struggled with cultural holidays and traditions in respect to having their child on home PN\(^7\).

The format of the focus group involved researchers posing framed questions which may create a researcher bias. There was also bias involved in the presence of the facilitators as they were known to the participants. This may have impacted the results of the focus group as the families may want to show gratitude and not want to appear ungrateful\(^5\).

The qualitative analysis was completed by a co-investigator involved in the focus group and then reviewed by the primary facilitator and co-facilitator. There was a risk of bias and error in the choice of descriptive terminology used to describe the results as well as a possible reliability fallacy in which one makes a mistake in the logic of their analysis.
6.9 Summary

Children with intestinal failure have experienced improved survival and as a result, more children are going home on parenteral nutrition. An understanding of their QOL is essential in preparing families for going home on PN, assessing treatment effects and options as well as in the coordination of support for patients and their families. In order to help reconcile conflicting evidence in understanding QOL of children on home PN, as suggested by Rosenbaum, we used an existential approach by obtaining personal perspectives, where possible, as these evaluations are important for QOL assessment regardless of observed function or limitations. Parent and child views are essential because they may conflict with what the clinician’s main concerns are; for example, the parent is upset with the delivery of the PN solution versus the clinician’s concern of the child’s micronutrient status. This was the first study that we were aware of that described QOL in children on home PN using a mixed-methods approach which allowed us to gain insight that may not have been possible with one method, as each have limitations. It was also the first pediatric study to directly compare the QOL of children on home PN with those who have had a SBTx. There was published data in adults describing QOL on home PN compared to the SBTx population however children are unique in terms of biologics, development, psychological and social aspects and thus it is not appropriate to extrapolate adult data directly to children.

Results of this study demonstrated that via proxy assessment of children on home PN, there was a compromised QOL compared to healthy published norms. This was evident in quantitative domains for physical, emotional, social and school functioning, functional status as well as perceptions of their general health and concern of global health in the present and future. However in the lived experience, the parents described that they were content with their child’s QOL. The parents expressed being able to enjoy life with their child and entire family and had an appreciation and gratitude for the small things in life. There were numerous challenges that the families faced in having a child who required home PN including social and emotional impacts. There is a true need for support for these families and their children on home PN that they felt would be best served by contact with other families who had similar experiences.
The children on home PN had a perception that their functioning and QOL was comparable to other children their age except in regards to their general health, potentially future health and recognition that their therapy impacted their family. They also scored themselves lower on the PedsQL™ than the normative population in terms of physical, social, emotional and school functioning. In comparison to children surviving their SBTx, there appeared to be a very similar experienced QOL with the potential of more pain experienced and a worsened overall view of their general health on home PN.

The small sample size prevented assessment of clinical variables related to home PN and medical therapy that may have contributed to QOL for both parent and proxy assessment. This needs to be investigated further.

There has been discussion and debate regarding the appropriateness of including QOL as an indication for SBTx over long term home PN. At present, the mortality rate for SBTx is much higher than that of patients on home PN. The survival rate for children on home PN is 90% and is increasing with improvement in care and advancement of medical therapy. Currently, survival outcomes for SBTx ranges from 78-85% at 1 year and 56-61% at 5 years, however these outcomes are improving. In addition, as suggested by Sudan et al., earlier transplantation would not be a better option if patients were experiencing renal failure, infection, malignancy, graft dysfunction and frequent hospitalizations post SBTx. The results of this study do show that the QOL was similar between the two groups although, interpretation is cautioned as the SBTx were all transplant survivors. A longitudinal study of pre and post transplant would provide a better understanding of how SBTx and cessation of home PN impacts QOL.

In summary, a mixed-methods approach to understanding home PN was essential in providing a more holistic picture of the children’s and family’s lives and what it meant to be and have a child on home PN. As Rosenbaum stated: “It is important to separate the disorder from the person, and not make assumptions about what life must be like for that child or their family.”

6.10 Future Directions

The development of a disease-specific home PN module may assist in capturing more relevant treatment and disease-specific issues. This could act as a complementary tool to a generic QOL
questionnaire to continue to allow comparison to norms and to capture multidimensional domains that apply to all families and not just those with chronic illness. Taylor et al. published an adolescent liver transplant model that included both patient and disease related variables. This model was an example of how issues such as age of home PN initiation, impact of home PN on fatigue, family activities, side effects of home PN and other disease-related symptoms could be included in a disease-specific survey\textsuperscript{47}.

Successful discharge on home PN requires commitment from the multidisciplinary team, the family, thorough training, careful discharge planning and ongoing support for the family and child\textsuperscript{178}. Data from this study could be used to enhance the support programs available for these families and to offer them an opportunity to connect with one another. In addition, the development of a resource made for families and by families with information regarding challenges and preparation for home PN may benefit those going home on PN in the future.

A longitudinal assessment of families on home PN will help to capture more aspects of this dynamic construct of QOL and give a better idea of how QOL changes over time. It may also identify what life experiences are associated with changes in QOL\textsuperscript{45}. In addition, child interviews or further focus groups may provide a more thorough overview of the lived experience of having a child or being a child on home PN.

Overall, there is significant opportunity to advance the care and support through the understanding of child and family experiences on home PN. This data is the first step in appreciating the challenges and experiences of the intestinal failure survivors on home PN in comparison to healthy children and those surviving children post SBTx.
Appendices
## Appendix A
Summary of QOL Literature in Pediatric and Adult Home PN

### Pediatric Home PN

<table>
<thead>
<tr>
<th>Study, Author, Year</th>
<th>Methodology</th>
<th>Subjects</th>
<th>Tools Utilized</th>
<th>Key Findings</th>
</tr>
</thead>
</table>
| Letters/Notes: Quality of life of parents of children on home parenteral nutrition (study unpublished) | Cross-sectional, mixed methods study including focus groups, semi-structured interviews and quantitative surveys (Manchester, UK) | 11 parents of children on home PN; 11 parental controls who were matched for having children of same age (BANS only) | Qualitative
  - Focus Group
  - Semi-structured interviews | Parents expressed feeling physically tired, having difficulties taking holidays, shopping and spending time with their partners
Quantitative
  - General Health Questionnaire (GHQ-28)
  - British Artificial Nutrition Survey (BANS) | Admitted to feeling frustrated, annoyed, stressed and having difficulty sleeping
Proxy | GHQ-28 showed 7 of 11 parents exceeded threshold for psychiatric morbidity
BANS showed significant deterioration in family, sex, social life, and work life when compared to controls |
| [Home parenteral nutrition in children: Influence of initial prognosis on mother's experience.] | Prospective, quantitative, single-centred, pilot study (France) | 10 mothers of children on home PN (mean age of children was 2 years and 2 mos of age) | Max Hamilton Anxiety Scale (administered at 3 time points: pre-diagnosis, during teaching for home PN, 2-6 months after being home on PN) | Mother's anxiety was the highest at the point of diagnosis and was expressed as anxious humour, insomnia, and depression. Anxiety was reduced once they were trained in delivery and was minimal at follow-up visits when they demonstrated more confidence with PN technique
Increase in anxiety associated with child having a poor prognosis (e.g. estimated duration of home PN greater than 1 year) | A negative impact on anxiety included job renunciation and an unsettled future
Continued care and close follow-up had a positive impact on confidence and anxiety levels of mothers |
| Psychological Distress Associated With Home Parenteral Nutrition in Swedish Children, Adolescents, and Their Parents; Preliminary Results | Cross-sectional, quantitative study (Stockholm, Sweden) | 21 families (proxy respondents) of children on home PN with mean age of 7 years (range from 3-15 yrs) | Home PN procedures (non-validated) questionnaire with closed and open questions Child Behaviour Checklist (CBCL) questionnaire Self Assessment – Proxy | 13 children had external caregivers. 25% of families had someone else that they could rely on to assist with home PN
Significantly lower scores on CBCL total social competence scale including activities, social interaction, and school when compared to norms
CBCL total problem score was significantly higher than published norms especially in internalizing symptoms; externalizing symptoms was not statistically different
Parents felt children demanded high level of attention, were anxious, shy and sensitive with changing emotions and complained of non-specific bodily pain
Parents noted problems with eyes and skin, poor coordination and speech | Scores found to be similar to inflammatory bowel disease children but worse than children with diabetes and chronic tension headache |
<table>
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<tr>
<th>Study Title</th>
<th>Methodology</th>
<th>Participants</th>
<th>Results</th>
</tr>
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| The Lived Experience of Home Total Parenteral Nutrition: An Online Qualitative Inquiry with Adults, Children, and Mothers<sup>67</sup> | Semi-structured, qualitative open-ended, in-depth interviews conducted online via instant messaging (Nashville TN, USA) | 6 adults on home PN, 3 children on home PN (aged 8-10 years), 3 mothers of the children on home PN | No statistically significant difference in social support total score and subscale adequacy of social integration compared to norms  
Significantly lower score on subscales of availability of and adequacy of attachment  
Significantly higher score on subscale availability of social integration  
Pressure on families was assessed by single question related to alcohol abstinence and 10 of 21 parents abstain from alcohol due to home PN  
6 major themes emerged: affirmation of life, infusion-related complications, lifestyle adaptations, self-worth for adults, isolation, and food intake; 2 minor themes were related to inadequate information from healthcare providers and cost of home PN  
Children and mothers expressed fears regarding the complications of being on home PN (development of IFALD, central line infections, loss of venous access)  
Children reported participating in similar activities as peers  
Children felt main difference from peers was related to their growth (each were shortest in their class)  
Children reported pressure and stress to eat in social situations where eating was central theme (e.g. holidays)  
Hunger was a critical issue for adults but not for children  
Mothers expressed that home PN allowed their child a chance at life  
Mothers felt overwhelmed in terms of care requirements and the initial period of home PN  
Mothers felt it was stressful to find sources of support for themselves |
| Satisfaction in Different Life Domains in Children Receiving Home Parenteral Nutrition and Their Families<sup>68</sup> | Cross-sectional, quantitative, national, multicenter study (5 pediatric institutions in France) | 72 pediatric patients on home PN, 90 siblings, 67 fathers, 69 mothers | Proxy – Patients & Siblings  
Qualin questionnaire (<3 years of age)  
Auquei questionnaire (3-5 years of age and 6-11 years of age)  
Self Assessment – Children  
OK.ado questionnaire (≥12 years of age)  
Proxy – Proxy  
Subjective Quality of Life Profile  
Overall QOL scores high in all age groups and not significantly different than reference normal population  
<3 years of age had significantly lower scores in health, eating, and speaking  
3-11 years of age had significantly lower scores in items related to hospital, health, doctors, medication, obligations, and becoming a grown-up  
No significant differences between siblings and patients except that siblings had higher satisfaction scores for eating and health  
Children significantly higher QOL scores than health adolescents, also higher in school and relations with adults  
Significantly lower scores in world events, doctors, medications, health, sports  
Compared to siblings, there were no significant differences  
Mothers had significantly lower scores on QOL compared to reference norms  
Mothers had lower satisfaction scores than fathers relating to work, inner life, and freedom  
Mothers scores not significantly associated with QOL scores of children |
### Associations
- Presence of stoma had positive impact on QOL for children <3 years of age and no impact on QOL for children aged 3-11 years
- Presence of stoma had negative impact on QOL relative to social life and the future

**Review of homecare packages for paediatric HPN patients**

Sexton et al. 2005

- Cross-sectional, qualitative, semi-structured, recorded interviews (Birmingham, UK)
- 20 families (over half were mothers only), some fathers were present and 3 older children were included. Mean age 3 yrs (6 months to 17.5 yrs); n=12 were >=3 yrs, n=8 were < 3 yrs
- Semi-structured (open and closed) questions in person (2 on the telephone)
- 3 main themes: physical, psychological and burden of care
  - Physical exhaustion was related to sleep disruption and managing symptoms related to intestinal function (e.g. diarrhea, vomiting)
  - Psychological issues related to having homecare which intruded on their private lives however others felt that homecare had a positive impact for both practical and psychological support
  - Parents expressed isolation and a strong burden of care in taking care of these children
- Described impact both emotionally and financially
- Described challenges of communication with medical teams
- Parents described fearing for their child’s future and the impact of the situation on the families

### Adult Home PN

<table>
<thead>
<tr>
<th>Study, Author, Year</th>
<th>Methodology</th>
<th>Subjects</th>
<th>Tools Utilized</th>
<th>Key Findings</th>
</tr>
</thead>
</table>
| **Assessing the quality of life of patients with intestinal failure on home parenteral nutrition**<sup>75</sup>  
Richards et al. 1997 | Cross-sectional, quantitative, single-centre study (Manchester, UK) | 51 adult patients on home PN (mean age of respondents not available) | Short Form 36 Health Survey (SF-36) to assess QOL  
EuroQOL Health State utility scores assessing QOL | Significantly lower scores for home PN patients compared to norms in terms of physical function, role related to physical function, bodily pain, general health, vitality, and social functioning; no difference in terms of role related to emotional function and mental health.  
Patients on home PN rated their health significantly lower on the EuroQOL compared to norms  
Younger respondents (<45 years) were significantly better than the older respondents (>55 years) in terms of physical functioning, social functioning and emotional role  
5 patients addicted to narcotic analgesics had SF-36 domain scores 25% lower than the other home PN patients however this was not statistically different |
| **Quality of life in patients receiving home parenteral nutrition**<sup>76</sup>  
Jeppesen et al. 1999 | Cross-sectional, quantitative, single centre study (Copenhagen, Denmark) | 49 adult patients with intestinal failure on home PN (mean age 45.4 yrs; range 37.7 to 56.9 yrs)  
36 adult patients with anatomical or functional | Sickness Impact Profile (SIP) measuring patient perception of activity performance in everyday life  
Inflammatory Bowel Disease Questionnaire (IBDQ) for disease specific for IBD to measure subjective health status | Home PN patients scored significantly lower on the SIP overall impact and QOL scores; lower in all specific areas including mobility, body care, social interaction, emotions, communication, sleep and rest, eating, work, home management, and recreational activities  
Home PN patients scored significantly lower on IBDQ overall score and on certain subscales; lower scores in relation to having more loose stools, abdominal pain, and nausea; increase in overall systemic symptoms including fatigue and poor sleeping; lower overall emotional function including less satisfaction and more anger due to bowel disease; worsened overall social function  
Females scored lower than males and older individuals (>45 years) had lower scores than younger respondents |
<table>
<thead>
<tr>
<th>Study</th>
<th>Design</th>
<th>Setting</th>
<th>Participants</th>
<th>Measures</th>
<th>Findings</th>
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<tr>
<td><strong>Longitudinal Assessment of Outcome, Health Status, and Changes in Lifestyle Associated With Long-Term Home Parenteral and Enteral Nutrition</strong>&lt;sup&gt;77&lt;/sup&gt;</td>
<td>Prospective, quantitative, single-centre study (Albany NY, USA)</td>
<td>Short bowel not on home PN (mean age 50.0 yrs; range 44.1-60.4 yrs)</td>
<td>• No significant differences were noted in relation to presence of a stoma, diagnosis, nor duration of home PN</td>
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<td>Malone M. 2002</td>
<td>17 adult patients (13 on home PN and 4 on home enteral nutrition (EN)); mean age of home PN at start was 54.8 yrs and 50.3 yrs in home EN sample</td>
<td>Short Form 36 Health Survey (SF-36) assessing QOL</td>
<td>SF-36 scores were significantly lower than population norms at both time points in all categories</td>
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<td>Lifestyle Symptom Questionnaire (frequency of symptoms and impact of PN and EN on lifestyle)</td>
<td>There were no significant differences in the SF-36 between the home PN and EN group and scores did not change significantly over the 3 year time period; there were no differences in age groups when separated between patients &lt; and &gt; 50 years of age</td>
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<td></td>
<td>Studied 2 time points, 3 years apart</td>
<td>Non significant but prevalent issues related to lifestyle and activity including sleep disruption, difficulties with travel and limitations in social life and leisure activities and choices of entertainment</td>
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<td></td>
<td>Most common clinical complaints in Home PN group include fluid retention, thirst and frequent urination</td>
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<td><strong>Problems Experienced by Patients Receiving Parenteral Nutrition at Home: Results of an Open Interview Study</strong>&lt;sup&gt;73&lt;/sup&gt;</td>
<td>Cross-sectional, qualitative, dual-centred, open interview study (Amsterdam, The Netherlands)</td>
<td>48 adults (mean age 52; range 29-82 years)</td>
<td>• 7 central themes including negative emotions (anxiety, isolation, fear), physical problems (e.g. fatigue, nausea, diarrhea), social limitations, dependence on others, incapability, complications, and problems with care providers</td>
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<td>Huisman–de Waal et al. 2006</td>
<td>4 open ended questions to assess nature and context of problems experienced by patients on home PN</td>
<td>4 main concepts and themes including the terminology to describe home PN therapy, PN as lifesaving therapy, lifestyle adaptation, quality of life (being at home), sustenance and security and negative issues related to food and eating</td>
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<td><strong>A pilot study of a qualitative interview guide for quality of life in home parenteral nutrition patients</strong>&lt;sup&gt;74&lt;/sup&gt;</td>
<td>Cross-sectional, qualitative, pilot study (Providence RI, USA)</td>
<td>3 adults (aged 22, 42 and 72 yrs) on home PN for fewer than 3 years (convenience sample)</td>
<td>• 6 main concepts and themes including the terminology to describe home PN therapy, PN as lifesaving therapy, lifestyle adaptation, quality of life (being at home), sustenance and security and negative issues related to food and eating</td>
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<td>Winkler et al. 2006</td>
<td>Semi-structured interview guide consisting of background questions and feelings and experiences related to PN, QOL and meaning of food</td>
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### Appendix B
Summary of QOL Literature in Pediatric and Adult Post SBTx

#### Pediatric Post SBTx

<table>
<thead>
<tr>
<th>Study, Author, Year</th>
<th>Methodology</th>
<th>Subjects</th>
<th>Tools Utilized</th>
<th>Key Findings</th>
</tr>
</thead>
</table>
| **Parental Psychosocial Outcomes in Pediatric Liver and/or Intestinal Transplantation: Pretransplantation and the Early Postoperative Period**<sup>35</sup> | Cross-sectional, quantitative, single centred, pilot study (Pittsburgh PA, USA) | • 42 families of liver and intestinal transplant recipients (including 61 parents – 41 mothers and 20 fathers)  
• Sampled at two time points: Pre-transplantation (n=30 parents) and 2 months post transplantation (n=31 parents) | **Parental Psychosocial Outcomes**  
• Brief Symptom Inventory (BSI) to measure mental health  
• Parenting Stress Index (PSI) to measure stress in parent-child relationship  
• Short Form-36 Health Survey (SF-36) to measure parental QOL | **Parental Psychosocial Outcomes**  
• Parents demonstrated clinically significant psychological symptoms on global index of distress from the BSI with fathers rating higher levels of distress than moms; no significant difference between parents pre and post transplant  
• No significant differences in parenting stress compared to published norms in either time period  
• Significantly lower scores on the vitality subscale of the SF-36 compared to norms however ranked themselves higher in terms of physical functioning; differences not presented between the two time points | **Predictors of Parental Psychosocial Outcomes**  
• Family environment and conflict was significantly associated with worsened parental mental health on BSI  
• Male sex, a younger child and a greater number of persons in the household was associated with higher parenting stress scores on PSI  
• A younger child was associated with better parental physical functioning on the SF-36 and a lower global perception of well-being |
| **Neurodevelopmental outcomes of infant intestinal transplant recipients**<sup>81</sup> | 4 cross-sectional, quantitative, single centre studies (Miami FL, USA) | **Study #1** | Bayley Scales of Infant Development II (BSID II) for children 1 to 42 months of age  
- MDI (mental development index)  
- PDI (psychomotor development index)  
**Differential Abilities Scale (DAS)** a battery of cognitive tests for children aged 2 years 6 months to 17 years 11 months of age | **Study #1** | Infants undergoing intestinal/multivisceral transplantation show significantly more cognitive delays than infants undergoing single-organ liver transplantation  
No infants undergoing any type of transplant with normal motor functioning  
**Study #2** | Infants who had received a transplant during infancy may also suffer severe motor delays, none were considered within normal limits  
Infants post SBTx/multivisceral transplant had more cognitive delays than children who had received an isolated liver transplant  
**Study #3** | Most infants studied pre and post multivisceral transplant were significantly delayed in both cognitive and motor functioning  
**Study #4** | Half of the children were still significantly delayed in mental and motor development 6 to 20 months post transplant  
2 preschool aged children were in very low range of conceptual ability and 2 were considered below average |
|---|---|---|---|---|---|---|---|---|
| Thevenin et al. 2004 | **Study #1** | 27 infants pre liver or intestinal/multivisceral tx (age 5 to 40 mos) | **Proxy** | Child Health Questionnaire Parent Form 50 (CHQ-PF50)  
**Self Assessment – Children** | Child Health Questionnaire Child Form 87 (CHQ-CF87) | **Proxy** | Child Health Questionnaire Parent Form 50 (CHQ-PF50)  
**Self Assessment – Children** | Child Health Questionnaire Child Form 87 (CHQ-CF87) | **Proxy** | Child Health Questionnaire Parent Form 50 (CHQ-PF50)  
**Self Assessment – Children** | Child Health Questionnaire Child Form 87 (CHQ-CF87) | **Proxy** | Child Health Questionnaire Parent Form 50 (CHQ-PF50)  
**Self Assessment – Children** | Child Health Questionnaire Child Form 87 (CHQ-CF87) |
| **Study #2** | 23 infants post liver or intestinal/multivisceral tx (age 7 to 47 mos) | **Differential Abilities Scale (DAS)** a battery of cognitive tests for children aged 2 years 6 months to 17 years 11 months of age | **Study #2** | Infants who had received a transplant during infancy may also suffer severe motor delays, none were considered within normal limits  
Infants post SBTx/multivisceral transplant had more cognitive delays than children who had received an isolated liver transplant  
**Study #3** | Most infants studied pre and post multivisceral transplant were significantly delayed in both cognitive and motor functioning  
**Study #4** | Half of the children were still significantly delayed in mental and motor development 6 to 20 months post transplant  
2 preschool aged children were in very low range of conceptual ability and 2 were considered below average |
| **Study #3** | 5 infants pre & post multivisceral tx (age 5 to 26 mos) | **Study #3** | Most infants studied pre and post multivisceral transplant were significantly delayed in both cognitive and motor functioning  
**Study #4** | Half of the children were still significantly delayed in mental and motor development 6 to 20 months post transplant  
2 preschool aged children were in very low range of conceptual ability and 2 were considered below average |
| **Study #4** | 10 children post intestinal/multivisceral tx (14 mos to 8 yrs) | **Study #4** | Half of the children were still significantly delayed in mental and motor development 6 to 20 months post transplant  
2 preschool aged children were in very low range of conceptual ability and 2 were considered below average |

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**Quality of Life after Pediatric Intestinal Transplantation: The Perception of Pediatric Recipients and Their Parents**<sup>18</sup>  
Sudan et al. 2004  
Cross-sectional, single centre quantitative analysis of QOL  
Proxy assessment n=22 parents (mean age of children not reported however all children between 5-18 yrs)  
Self Assessment n=21 children with mean age of 11 yrs

| **Proxy** | Child Health Questionnaire Parent Form 50 (CHQ-PF50)  
**Self Assessment – Children** | Child Health Questionnaire Child Form 87 (CHQ-CF87) | **Proxy** | Child Health Questionnaire Parent Form 50 (CHQ-PF50)  
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**Self Assessment – Children** | Child Health Questionnaire Child Form 87 (CHQ-CF87) | **Proxy** | Child Health Questionnaire Parent Form 50 (CHQ-PF50)  
**Self Assessment – Children** | Child Health Questionnaire Child Form 87 (CHQ-CF87) |

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**Proxy** assessments directly compared to published norms.  
Comparison of liver transplant recipients and children with diabetes with published norms presented  
Self assessment comparisons included published norms and children with end stage renal disease (ESRD)  
Upon comparison of proxy and self assessments, only significantly different scores in general health perceptions and physical role limitations, with lower proxy scores

**Proxy**  
Proxies rated children significantly lower than published norms in physical functioning, role/social limitations related to physical function, general health, family activities, and parental impact on both emotion and time  
Separating age groups parent proxy scored children between ages of 5-10 significantly lower than older children (10-18 yrs) in regards to global health, general health perception and family activities

**Self Assessment – Children**  
No significant differences in children’s self assessment compared to published norms  
Children post-SBTx scored significantly higher in mental health compared to both norms and children with ESRD on dialysis.
<table>
<thead>
<tr>
<th>Study Title</th>
<th>Study Design</th>
<th>Participants</th>
<th>Instruments/Outcomes</th>
</tr>
</thead>
</table>
| Impact of Paediatric Home Parenteral Nutrition and Intestinal Transplantation on Parental Mental Health: Abstract International Small Bowel Transplant Symposium | Prospective, single-centre, quantitative study on QOL and mental health in parents of children in the first year following SBTx (Birmingham, UK) | Group 1: Parents of children at 3 time points (pre SBTx assessment, 6 mos and 12 mos post SBTx); n=12  
Group 2: Parents of children on home PN; n=4 | Child Health Questionnaire Parent Form 50 (CHQ-PF50)  
General Health Questionnaire (GHQ)  
Quality of Life After Pediatric Intestinal Transplantation: Outcomes and Predictors: Abstract International Small Bowel Transplant Symposium  
Prospective, single-centre, quantitative study on QOL and mental health in parents of children in the first year following SBTx (Birmingham, UK) | Parents of children awaiting a SBTx had lower GHQ scores (significance not stated) compared to parents of kids on home PN related to increase in somatic symptoms, anxiety/insomnia, social dysfunction and depression  
GHQ scores improved post SBTx  
CHQ-PF50 scale of parental impact on emotion improved post SBTx however there was no improvement on parental impact on time |
| Quality of Life After Pediatric Intestinal Transplantation: Outcomes and Predictors: Abstract International Small Bowel Transplant Symposium | Cross-sectional, single-centre, quantitative analysis of QOL (Los Angeles CA, USA) | 33 parents of children post intestinal transplant | Child Health Questionnaire Parent Form 50 (CHQ-PF50) of QOL  
PedsQL 4.0™ Proxy Report of QOL | Compared to published data:  
CHQ PF-50 compared to published norms (n=391) and pediatric liver transplant (n=35)  
PedsQL 4.0™ Proxy forms compared to published norms (n=718) and pediatric liver transplant (n=77)  
Overall, proxy ranked children lower on 76% of QOL domains compared to healthy norms and 41% of domains compared to liver transplant  
Proxy assessed significantly lower scores on the CHQ-PF50 compared to norms in relation to physical function, physical role, general health, emotional role, behaviour, parental impact on time and emotions and impact on family activities  
Compared to norms, assessed significantly lower scores on PedsQL 4.0™ on physical, emotional, social and school functioning as well as psychosocial health summary scale  
Associations  
Using CHQ-PF50 as primary outcome, predictors of low QOL included single care provider, worse nutrition and renal status, the presence of devices (e.g. feeding tube, ostomy, or central line) and an intravenous fluid or PN requirement  
Using the PedsQL 4.0™ as primary outcome, having an IV fluid or PN requirement was related to lower QOL scores |
<table>
<thead>
<tr>
<th>Study, Author, Year</th>
<th>Methodology</th>
<th>Subjects</th>
<th>Tools Utilized</th>
<th>Key Findings</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Quality of Life After Small Intestinal Transplantation and Among Home Parenteral Nutrition Patients</strong>&lt;sup&gt;87&lt;/sup&gt; DiMartini et al. 1998</td>
<td>Cross-sectional and retrospective, quantitative, single centre study (Pittsburgh PA, USA)</td>
<td>• 9 adults post SBTx (mean age 26.6 yrs; range not provided)</td>
<td>• Quality of Life Inventory (QOLI) validated in adult liver transplant patients only (disease-specific)</td>
<td><strong>Post SBTx Group</strong>&lt;br&gt;• Comparing pre-PN and during home PN status - significantly worsened aspects in most areas of lives after starting home PN except for marital relationships, medical compliance and medical satisfaction&lt;br&gt;• Comparing during PN to post SBTx, they reported significant improvements in most areas of QOL: less anxiety, less depression, better mental status, decreased stress, improved optimism, less impulsiveness, improved control, increased sexuality, improved coping, better mobility and appearance, decreased gastrointestinal symptoms, improved sleep and energy, ability to participate in recreational activities, improved social support and quality of relationships; no changes in alcohol and drug use, cognitive and emotional style, pain and discomfort, finances, marital relationships, medical compliance and satisfaction and parenting&lt;br&gt;• Comparing post SBTx to pre-PN state, most areas were compared favourably however the following were worse: greater need for medications, decreased mobility, increased pain and discomfort, difficulty parenting, poorer sleep, loss of control and poor quality of social support&lt;br&gt;&lt;br&gt;<strong>Home PN Group</strong>&lt;br&gt;• Comparing pre-PN to current home PN state, there was significantly reported worsening in many domains including increased anxiety, depression, drug use, pain and discomfort, stress, digestive and urinary symptoms, loss of control and decreased physical mobility, energy, optimism, and sleep</td>
</tr>
<tr>
<td><strong>Quality of Life of Patients After Intestinal Transplantation</strong>&lt;sup&gt;88&lt;/sup&gt; Rovera et al. 1998</td>
<td>Prospective/retrospective/cross-sectional, quantitative, single-centre trial (Pittsburgh PA, USA)</td>
<td>• 10 adults post SBTx (mean age 34.2 yrs; range 23-62 yrs)</td>
<td>• Quality of Life Inventory (QOLI) validated in adult liver transplant patients only (disease-specific)</td>
<td><strong>Statistically significant differences between post SBTx and home PN groups in terms of drug use and medical compliance with worsened scores for the post SBTx group; no other significant differences between the two groups in the remaining 23 domains</strong>&lt;br&gt;• 4 patients in SBTx group who repeated the questionnaires 2 years apart demonstrated statistically significant improvements over time in terms of anxiety, sleep and impulsiveness/control&lt;br&gt;• 4 patients in home PN group who repeated the questionnaires 2 years apart demonstrated a statistically significant improvement over time in terms of finances however a worsening in the mental status</td>
</tr>
<tr>
<td>Study Title</td>
<td>Study Design</td>
<td>Participants</td>
<td>Measures</td>
<td>Findings</td>
</tr>
<tr>
<td>---------------------------------------------------------------------------</td>
<td>-----------------------------------</td>
<td>------------------------------------------------------------------------------</td>
<td>--------------------------------------------------------------------------</td>
<td>-------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------------</td>
</tr>
</tbody>
</table>
| Quality of Life in Adults Following Small Bowel Transplantation<sup>81</sup> Cameron et al. 2002 | Cross-sectional, quantitative, dual-centre study (Cambridge, UK) | 3 groups of adult patients                                                   | Nottingham Health Profile (NHP) measuring QOL; Short Form 36 Health Survey (SF-36) measuring QOL | • No statistically significant differences on NHP and SF-36 comparing the post SBTx group and the stable home PN group  
• Comparing patients post SBTx and those with complicated intestinal failure there were significantly lower scores on NHP in terms of energy, social isolation, emotional reactions and physical function; lower scores were present on SF-36 for emotional roles, social functioning and general health  
• Comparing patients stable on home PN with complicated intestinal failure, there were significantly lower scores on the NHP in terms of energy, sleep, social isolation, physical mobility, emotional reactions and physical function; on the SF-36, there were significantly lower scores on the physical and emotional role domains as well as social functioning |
| Quality of Life on Home Parenteral Nutrition or After Intestinal Transplantation<sup>49</sup> Pironi et al. 2006 | Cross-sectional, quantitative, single-centre study (Bologna, Italy) | 18 adult patients on home PN and stable (mean age 46.9 yrs); 12 adult patients post-SBTx (mean age 35.7 yrs) | Short Form 36 Health Survey (SF-36) to assess QOL | • Statistically significant difference in domain of bodily pain which was worse in the home PN group  
• No other significant differences although trend of home PN patients scoring lower in all other domains |
| Nutrition and Quality of Life Following Small Intestinal Transplantation<sup>90</sup> O’Keefe et al. 2007 | Prospective, quantitative, single-centre study (Pittsburgh PA, USA) | 46 adult patients pre-SBTx on home PN and then post SBTx (median age 44 yrs); Quality of life instrument not specified in study (likely the QOLI disease specific tool validated in the liver tx population); Subjective global | | • Overall global QOL assessment was significantly lower in patients pre-tx on home PN in comparison to those stable on home PN; there was a significant improvement from pre to post SBTx periods  
• On the QOL instrument, there were no significant differences between pre SBTx and those stable on home PN however, there was a significant improvement from pre to post SBTx in terms of anxiety, depression, cognitive emotion, appearance, stress, parenting, digestive and urinary function, optimism, control, medical compliance, quality of relationship and social relations, leisure and recreation |
<table>
<thead>
<tr>
<th>Psychological Adaptation and Quality of Life of Adult Intestinal Transplant Recipients: University of Bologna Experience</th>
<th>Cross-sectional, quantitative, single-centre study (Bologna, Italy)</th>
<th>Adult SBTx recipients (n=27)</th>
<th>Psychological Well-Being (PWB) Scales</th>
<th>Psychological Well-Being (PWB) Scales</th>
<th>Psychological Well-Being (PWB) Scales</th>
</tr>
</thead>
<tbody>
<tr>
<td>Abstract International Small Bowel Transplant Symposium 92 Golferi et al. 2009</td>
<td>Healthy, controls (n=27)</td>
<td>World Health Organization Quality of Life (WHOQOL)Brief</td>
<td>Symptom Questionnaire (SQ)</td>
<td>WHOQOL score for the psychological domain was significantly lower in SBTx group</td>
<td>WHOQOL score for the psychological domain was significantly lower in SBTx group</td>
</tr>
<tr>
<td>13 adult patients on stable home PN (median age 61 yrs; range 31-80 yrs)</td>
<td>range 22-66 yrs)</td>
<td>assessment scale from 1 to 10 where life was wonderful to intolerable</td>
<td>PWB scales demonstrated significantly higher scores in personal growth and lower scores in positive relations and autonomy</td>
<td>PWB scales demonstrated significantly higher scores in personal growth and lower scores in positive relations and autonomy</td>
<td>PWB scales demonstrated significantly higher scores in personal growth and lower scores in positive relations and autonomy</td>
</tr>
<tr>
<td>Psychological Well-Being (PWB) Scales</td>
<td>Symptom Questionnaire (SQ)</td>
<td>WHOQOL score for the psychological domain was significantly lower in SBTx group</td>
<td>WHOQOL score for the psychological domain was significantly lower in SBTx group</td>
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<td>WHOQOL score for the psychological domain was significantly lower in SBTx group</td>
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<td>WHOQOL score for the psychological domain was significantly lower in SBTx group</td>
<td>WHOQOL score for the psychological domain was significantly lower in SBTx group</td>
<td>WHOQOL score for the psychological domain was significantly lower in SBTx group</td>
</tr>
<tr>
<td>SBTx patients who had admissions &gt;8 demonstrated significantly higher scores in anxiety and depression on the SQ</td>
<td>SBTx patients who had admissions &gt;8 demonstrated significantly higher scores in anxiety and depression on the SQ</td>
<td>SBTx patients who had admissions &gt;8 demonstrated significantly higher scores in anxiety and depression on the SQ</td>
<td>SBTx patients who had admissions &gt;8 demonstrated significantly higher scores in anxiety and depression on the SQ</td>
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<td>SBTx patients who had admissions &gt;8 demonstrated significantly higher scores in anxiety and depression on the SQ</td>
</tr>
<tr>
<td>SBTx patients on Daclizumab protocol demonstrated significantly higher scores in depression and somatic symptoms of the SQ</td>
<td>SBTx patients on Daclizumab protocol demonstrated significantly higher scores in depression and somatic symptoms of the SQ</td>
<td>SBTx patients on Daclizumab protocol demonstrated significantly higher scores in depression and somatic symptoms of the SQ</td>
<td>SBTx patients on Daclizumab protocol demonstrated significantly higher scores in depression and somatic symptoms of the SQ</td>
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<td>SBTx patients on Daclizumab protocol demonstrated significantly higher scores in depression and somatic symptoms of the SQ</td>
</tr>
</tbody>
</table>
Appendix C
The Hospital for Sick Children Ethics Approval
The Research Ethics Board for The Hospital for Sick Children is organized and operates according to the principles and practices outlined in the Tri-Council Policy Statement, the ICH Harmonized Tripartite Guidelines: Good Clinical Practice, and Division 5 and the Medical Devices Regulations of the Food and Drug Act as well as the Natural Health Products Regulations of Health Canada. This signed document is in lieu of the Health Canada Research Ethics Board Attestation Form.

Approval & Terms of Agreement

Investigators: Dr. Paul Wales, M.Carricato, N.de Silva, J.Maxwell, S.Pollock-Bar Ziv, V.Ng

Study Title: Quality of Life in Pediatric Patients with Intestinal Failure on Home Parenteral Nutrition

REB File number: 1000014635 Level of Continuing Review: IB
Protocol Version Date: November 30, 2009
Consent & Assent Form Version Date(s): Subject and Control: Parent, Self, Assent, and Audio All: November 30, 2009
Investigator's Brochure Version Date: N/A
Other Approved Recruitment Document Dates: Case Report Form November 30, 2009, Focus Group Questions November 30, 2009, Questionnaire December 10, 2009

I agree to carry out the proposed research involving human subjects in accordance with the above-noted guidelines and regulations (as applicable) and using only the REB-approved study protocol and consent/assent form(s). I shall notify the division/department head and the REB prior to implementing any amendments in the protocol and consent/assent forms and of any deviations or any changes in study activity. I shall also notify the REB of any unexpected adverse events as per REB guidelines. As applicable, I certify that the research contract and corresponding protocol are consistent and will inform the contract manager of any protocol amendments as required.

I agree that, in accordance with the Personal Health Information Protection Act of Ontario, I am responsible for adhering to all conditions and restrictions imposed by the REB governing the use, security, disclosure, return and disposal of the research subjects' personal health information. I am also responsible for reporting immediately any privacy breaches to the REB Chair and to Janice Campbell, the Sick Kids privacy officer. I will ensure that the personal health information is used, only as necessary, to fulfill the specific research objectives and related research questions described in this application and approved by the REB.

Signature of Principal Investigator

I approve this research protocol, agree to share responsibility for its proper conduct, and will ensure that the REB is notified of concerns, as appropriate.

Signature of Division/Department Head

The REB of the Hospital for Sick Children has reviewed and approved the above-named research study.

Mr. Richard Sugarman, REB Chair
555 University Avenue, Toronto, Ontario, M5G 1X8
Tel: 416-813-6152 Fax: 416-813-5085 Email: richard.sugarman@sickkids.ca

DATE OF APPROVAL DEC 23 2009 EXPIRY DATE December 2010
Appendix D
Case Report Forms

Case Report Form #1 – Demographic Data
Quality of Life in Pediatric Patients with Intestinal Failure on Home Parenteral Nutrition

STUDY ID# ________________________
CONSENT RECEIVED TO PARTICIPATE:
YES
NO  REASON: __________________________

TO BE COMPLETED FOR ALL PATIENTS APPROACHED FOR STUDY

<table>
<thead>
<tr>
<th>Level of prematurity</th>
<th>__ weeks</th>
<th>N/A</th>
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</thead>
<tbody>
<tr>
<td>Age at time of assessment</td>
<td>__ weeks (if less than 1 year of age)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>__ months (between 1-2 years of age)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>__ years of age (&gt;= 2 years of age)</td>
<td></td>
</tr>
<tr>
<td>CORRECTED Age at time of assessment</td>
<td>__ weeks (if less than 1 year of age)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>__ months (between 1-2 years of age)</td>
<td></td>
</tr>
<tr>
<td></td>
<td>N/A</td>
<td></td>
</tr>
<tr>
<td>Gender</td>
<td>MALE  FEMALE</td>
<td></td>
</tr>
<tr>
<td>Primary Underlying Diagnosis</td>
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<td></td>
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<tr>
<td>Comorbidities</td>
<td></td>
<td></td>
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<tr>
<td>Presence of Ostomy?</td>
<td>Y  N</td>
<td></td>
</tr>
<tr>
<td>Presence of Drains?</td>
<td>Y  N</td>
<td></td>
</tr>
<tr>
<td>Number of Medications Daily</td>
<td>Total # ________________</td>
<td></td>
</tr>
<tr>
<td></td>
<td>Total # doses ________________</td>
<td></td>
</tr>
<tr>
<td>Current Enteral Nutrition Regimen</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Enteral Feeds?</td>
<td>Y  N</td>
<td></td>
</tr>
<tr>
<td>Feeding Tube?</td>
<td>NG  GT  NJ  GJ  None</td>
<td></td>
</tr>
<tr>
<td># hrs on feeds?</td>
<td>__________ hours</td>
<td></td>
</tr>
<tr>
<td>Oral Feeds?</td>
<td>Y  N</td>
<td></td>
</tr>
<tr>
<td>% total calories from EN?</td>
<td>_______%</td>
<td></td>
</tr>
<tr>
<td>Current Parenteral Nutrition Regimen</td>
<td></td>
<td></td>
</tr>
<tr>
<td># hrs of PN?</td>
<td>__________ hours</td>
<td></td>
</tr>
<tr>
<td>% of calories from PN?</td>
<td>_______%</td>
<td></td>
</tr>
<tr>
<td>Presence of Oral Aversion?</td>
<td>Y  N</td>
<td></td>
</tr>
<tr>
<td>GIFT PATIENTS ONLY</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Presence of IFALD?</td>
<td>C Bili &gt;100  YES  NO</td>
<td></td>
</tr>
<tr>
<td>Listed for Transplant?</td>
<td>Y  N</td>
<td></td>
</tr>
<tr>
<td>If yes, what type:</td>
<td></td>
<td></td>
</tr>
<tr>
<td>- Isolated Small Bowel</td>
<td></td>
<td></td>
</tr>
<tr>
<td>- Small Bowel/Liver</td>
<td></td>
<td></td>
</tr>
<tr>
<td>- Multivisceral</td>
<td></td>
<td></td>
</tr>
<tr>
<td>Duration of Transplant Listing</td>
<td>__________ days</td>
<td></td>
</tr>
<tr>
<td>Convert to number of days listed for transplant (=date of data collection – date of listing)</td>
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<td></td>
</tr>
<tr>
<td>Duration patient on home PN</td>
<td>__________ months</td>
<td></td>
</tr>
<tr>
<td>Or</td>
<td>__________ years</td>
<td></td>
</tr>
<tr>
<td>Age of HPN initiation</td>
<td>__________ months</td>
<td></td>
</tr>
<tr>
<td>Or</td>
<td>__________ years</td>
<td></td>
</tr>
</tbody>
</table>
Case Report Form #1 \( \text{Demographic Data} \)

Page 2

STUDY ID# ______________________

**SMALL BOWEL TRANSPLANT PATIENTS ONLY**

<table>
<thead>
<tr>
<th>Type of Transplant</th>
<th>- Isolated Small Bowel</th>
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</thead>
<tbody>
<tr>
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<td>- Small Bowel/Liver</td>
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<tr>
<td></td>
<td>- Multivisceral</td>
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<tr>
<th>Age at Time of First Transplant</th>
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<tbody>
<tr>
<td></td>
<td>__________ months (corrected age)</td>
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<td>__________ years</td>
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<table>
<thead>
<tr>
<th>Time Since Most Recent Tx (days)</th>
<th>__________ days</th>
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<tbody>
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<td>Convert to number of days since most recent transplant (=date of data collection – date of tx)</td>
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</table>

<table>
<thead>
<tr>
<th>Time Since Most Recent Tx (years)</th>
<th>&lt;1 year</th>
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<tbody>
<tr>
<td></td>
<td>1-3 years</td>
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<tr>
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<td>&gt;3 years</td>
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<table>
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<th>Retransplanted</th>
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</thead>
<tbody>
<tr>
<td></td>
<td>2=no</td>
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</table>

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<thead>
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<th># Retransplantations</th>
<th>1 Reason:</th>
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<tbody>
<tr>
<td></td>
<td>2 Reason:</td>
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<tr>
<td></td>
<td>3 Reason:</td>
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<table>
<thead>
<tr>
<th>Time Since Retransplant</th>
<th>1. __________ days</th>
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<tbody>
<tr>
<td></td>
<td>2. __________ days</td>
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<tr>
<td></td>
<td>3. __________ days</td>
</tr>
<tr>
<td></td>
<td>Convert to number of days listed for transplant (=date of data collection – date of retransplant)</td>
</tr>
</tbody>
</table>

PLEASE PROCEED TO NEXT PAGE ONLY IF PATIENT HAS PROVIDED CONSENT TO PARTICIPATE IN STUDY
**Case Report Form #2 – Additional Demographic Data**  
**Quality of Life in Pediatric Patients with Intestinal Failure on Home Parenteral Nutrition**  
**STUDY ID# ______________________**  
**FORM TO BE COMPLETED ONLY IF PATIENT HAS PROVIDED CONSENT TO PARTICIPATE IN STUDY**

<table>
<thead>
<tr>
<th>Use of anti-anxiety/antidepressant medications at follow-up visit</th>
<th>NOTES</th>
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</thead>
<tbody>
<tr>
<td>1 = yes (describe)</td>
<td></td>
</tr>
<tr>
<td>2 = no</td>
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</table>

<table>
<thead>
<tr>
<th>Number of recent hospitalizations (# in past 6 months)</th>
<th>NOTES</th>
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</thead>
<tbody>
<tr>
<td><strong><strong><strong># days as inpatient in past 6 months or other duration</strong></strong></strong>__</td>
<td></td>
</tr>
<tr>
<td><strong><strong><strong># admissions in past 6 months or other duration</strong></strong></strong>__</td>
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<table>
<thead>
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<th>Use of daily pain medication</th>
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<tbody>
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<td>2 = no</td>
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<table>
<thead>
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<th>Distance travelled to Sick Kids</th>
<th>NOTES</th>
</tr>
</thead>
<tbody>
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<td>1 = local GTA</td>
<td></td>
</tr>
<tr>
<td>2 = &lt;100 km from Sick Kids</td>
<td></td>
</tr>
<tr>
<td>3 = &gt;100 km from Sick Kids</td>
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</tr>
<tr>
<td>4 = Outside Ontario</td>
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</table>

<table>
<thead>
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<th>Patient education level</th>
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<tbody>
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<td>Describe</td>
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<table>
<thead>
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<th>Parental marital status</th>
<th>NOTES</th>
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</thead>
<tbody>
<tr>
<td>1=married</td>
<td></td>
</tr>
<tr>
<td>2=divorced</td>
<td></td>
</tr>
<tr>
<td>3=other</td>
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<table>
<thead>
<tr>
<th>Single or dual parent household income</th>
<th>NOTES</th>
</tr>
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<td>1=single parent</td>
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<tr>
<td>2=dual parent</td>
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<table>
<thead>
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<tr>
<td>2 = no</td>
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</table>
Appendix E
Consent Forms for Quantitative Arm of Study
Research Ethics Board

Retention of signed research consent forms;

The research consent form and assent form are a permanent part of the health record.

1. In-patient subjects: For in-patients enrolled in a study, the research consent form and assent form are to be retained on the patient health record. This form should be kept in the research section of the in-patient blue binder.

2. Out-patient subjects: For out-patients enrolled in a study, the research consent form and assent form should be sent to Health Records Department. Health Records staff will add it to the subjects health record in the correspondence section.

3. Non-Sick Kids subjects:
   a) For subjects who are patients at another institution, the research consent form and assent form are to be retained on that institution's health record.
   b) For subjects who are healthy volunteers e.g., normal controls, or subjects in psychological research based at schools or in the community, the research consent form and assent form should be retained by the primary investigator in their research files.

The Public Hospitals Act requires that all parts of the health record be retained for ten years past the patient's eighteenth birthday, or ten years past the last hospital contact if the patient is over the age of eighteen.

- For studies involving in-patients and out-patients, Health Records will assume responsibility for retaining records in accordance with the law.
- For studies involving patients at other institutions, the investigator should ensure that the forms are retained on that institution's health record.
- For studies involving healthy volunteers, the investigator must retain the forms.
Research Consent Form
(For those capable of providing consent)

Title of Research Project:
Quality of Life in Pediatric Patients with Intestinal Failure on Home Parenteral Nutrition

Investigators:
Paul Wales, MD General Surgery (416) 813-1490
Megan Carricato, RD MSc(c) Transplant Centre (416) 813-2119
Stacey Pollock-BarZiv, PhD Transplant Centre (416) 813-3604
Julia Maxwell, MSW Department of Social Work (416) 813-6195
Nicole deSilva, CNS-NP Division of Neonatology (416) 813-6736
Vicky Ng, MD Transplant Centre (416) 813-7733

Purpose of the Research:
Quality of life is an important outcome measure of intestinal failure and home parenteral nutrition dependence. We would like to get a better understanding of your quality of life while on home parenteral nutrition. This study will help us learn about your overall physical functioning, psychological and social well-being and general well-being.

Description of the Research:
The study will ask you to fill out a questionnaire package that will contain questions about your physical functioning, psychological well-being and social activities. These questionnaires are meant to assess your quality of life. The study asks you to fill out a questionnaire package once. These questionnaires will be completed during the regularly scheduled clinic appointment and are expected to take approximately 45 minutes to complete.

A review of your health record will be conducted to obtain important medical variables (such as age at home parenteral nutrition initiation, diagnosis, etc.)

If you are asked to and agree to participate in a 1-hour focus group with other children, you will be required to complete an additional consent form.

Potential Harms:
We know of no harm that taking part in this study could cause you.

Potential Discomforts or Inconveniences:
Discomforts or inconveniences associated with participation in this study include the time commitment required to complete the questionnaires. If you are asked to participate in the focus group, there will be an additional inconvenience in traveling to the hospital and a one hour time commitment to participate.
**Potential Benefits:**
This study will help to provide better care for some intestinal failure patients on home parenteral nutrition, as well as provide information about a comparison of quality of life pre and post small bowel transplant. This information is currently not available to professionals who care for these patients. This information will be used to improve the quality of the care provided to the intestinal failure population as a whole. Participants will receive a brief summary of the research findings.

**Confidentiality:**
We will respect your privacy. No information about who you will be given to anyone or be published without your permission, unless required by law. For example, the law could make us give information about you:
- If a child has been abused
- If you have an illness that could spread to others
- If you or someone else talks about suicide (killing themselves), or
- If the court orders us to give them the study papers.

Sick Kids Clinical Research Office Monitor or the regulator of the study may see your health record to check on the study. By signing this consent form, you agree to let these people look at your records. We will put a copy of this research consent form in your patient health record and give you a copy as well.

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During the focus group meeting we will remind everyone that the information shared is private and should not be repeated outside the group, but we cannot be sure that information shared in that setting will be kept private. People in groups may share information with others outside the group.

**Reimbursement:**
There will be no reimbursement for participation in the questionnaire portion of the study. Subjects and/or their guardians should not incur any out-of-pocket expenses, as the research will be conducted during a regularly scheduled clinic appointment.

However, if you agree to participate in the focus group, we will reimburse you for all of your reasonable out-of-pocket expenses up to a maximum of $20.00 (e.g. parking and getting you to and from Sick Kids on the day of the focus group).

**Participation:**
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New information that we get while we are doing this study may affect your decision to take part in this study. If this happens, we will tell you about this new information and we will ask you again if you still want to be in the study.

If you become ill or are harmed because of study participation, we will treat you for free. Your signing of this consent form does not interfere with your legal rights in any way. The staff of the study, and the hospital are still responsible, legally and professionally, for what they do.

**Sponsorship:**
The study is sponsored by the Principal Investigator and Sick Kids.

**Conflict of Interest:**
None of the research team members appear to have a conflict of interest to declare.

**Consent:**
By signing this form, I agree that:

1) You have explained this study to me. You have answered all my questions.
2) You have explained the possible harms and benefits (if any) of this study.
3) I know what I could do instead of taking part in this study. I understand that I have the right not to take part in the study and the right to stop at any time. My decision about taking part in the study will not affect my health care at Sick Kids.
4) I am free now and in the future, to ask questions about the study.
5) I have been told that my medical records will be kept private except as described to me.
6) I understand that no information about who I am will be given to anyone or be published without first asking my permission.
7) I agree to take part in this study.

__________________________________________
Printed name of subject                                    Subject’s signature & date

__________________________________________
Printed name of person who explained consent                Signature & date

__________________________________________
Printed witness’s name (subject does not read English)     Witness’s signature & date

If you have any questions about this study, please call Megan Carricato at (416) 813-2119.

If you have questions about your rights as a subject in a study or injuries during a study, please call the Research Ethics Manager at (416) 813-5718.

Page 3 of 3
Title of Study
Quality of Life in Pediatric Patients with Intestinal Failure on Home Parenteral Nutrition

Investigators:
Paul Wales, MD General Surgery (416) 813-1490
Megan Carricato, RD MSc(c) Transplant Centre (416) 813-2119
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Julia Maxwell, MSW Department of Social Work (416) 813-6195
Nicole deSilva, CNS-NP Division of Neonatology (416) 813-6736
Vicky Ng, MD Transplant Centre (416) 813-7733

Why are we doing this study?
We would like to get a better understanding of your quality of life while you are on home parenteral nutrition. This study will help us learn about your health and well-being, about how you are doing overall, about school, about your friends and family, about everyday activities that you do, and how you feel in general. This information will help us understand what life is like for our patients on home parenteral nutrition.

What will happen during the study?
You and/or your parent/guardian will be asked to fill out a questionnaire package one time during your regular follow-up clinic appointment. You may also be asked to participate in a 1 hour focus group.

Are there good things and bad things about the study?
This study may help us to make the care and services that we give to patients and families at Sick Kids better. There are no known harms from being in this study. The questionnaires might be a bit boring, but you will only need to fill them in once. The questionnaire package takes approximately 45 minutes to complete. If you have difficulty reading the questions or understanding the questions, someone will help you to go through them.

Who will know about what I did in the study?
No one will know that you were in the study. If we feel your health may be in danger, we may have to report your results to your doctor.
Can I decide if I want to be in the study?
You can decide if you want to be in this study. Nobody will be angry or upset if you do not want to be in the study. During the study, you can stop if you want. Please let us know if this is what you want. If you decide not to participate in the study, it will not change the care you receive at Sick Kids in any way. We are talking to your parent/guardian about the study and you should talk to them about it too.

**Assent:**
I was present when ___________________________ read this form and said that he or she agreed, or assented, to take part in this study.

______________________________  ____________________________
Printed name of person who obtained assent  Signature & Date
Title of Research Project:
Quality of Life in Pediatric Patients with Intestinal Failure on Home Parenteral Nutrition

Investigators:
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Purpose of the Research:
Quality of life is an important outcome measure of intestinal failure and home parenteral nutrition dependence. We would like to get a better understanding of your child’s quality of life while on home parenteral nutrition. This study will help us learn about your child’s overall physical functioning, psychological and social well-being and general well-being.

Description of the Research:
The study will ask you and/or your child to fill out a questionnaire package that will contain questions about their physical functioning, psychological well-being and social activities. These questionnaires are meant to assess your child’s quality of life. The study asks you and/or your child to fill out a questionnaire package once. These questionnaires will be completed during the regularly scheduled clinic appointment and are expected to take approximately 45 minutes to complete.

A review of your child’s health record will be conducted to obtain important medical variables (such as age at home parenteral nutrition initiation, diagnosis, etc.)

Potential Harms:
We know of no harm that taking part in this study could cause your child.

Potential Discomforts or Inconveniences:
Discomforts or inconveniences associated with participation in this study include the time commitment required to complete the questionnaires.

Potential Benefits:
This study will help the health care team to provide better care for intestinal failure patients on home parenteral nutrition, as well as provide information of a comparison of quality of life pre and post
small bowel transplant. This information is currently not available to professionals who care for these patients. This information will be used to improve the quality of the care provided to the intestinal failure population as a whole. Participants will receive a brief summary of the research findings.

**Confidentiality:**
We will respect your privacy. No information about who your child is will be given to anyone or be published without your permission, unless required by law. For example, the law could make us give information about you:
- If a child has been abused
- If you have an illness that could spread to others
- If you or someone else talks about suicide (killing themselves), or
- If the court orders us to give them the study papers.

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During the focus group meeting we will remind everyone that the information shared is private and should not be repeated outside the group but we cannot be sure that information about you will be kept private. People in groups may share information with others outside the group.

**Reimbursement:**
There will be no reimbursement for participation in the questionnaire portion of the study. Subjects and/or their guardians should not incur any out-of-pocket expenses, as the research will be conducted during a regularly scheduled clinic appointment.

However, if you and/or your child is asked and you agree/your child agrees to participate in a 1-2 hour focus group, you will be reimbursed for all your reasonable out-of-pocket expenses for attending the focus group to a maximum of $20.00 (e.g. parking and getting you/your child to and from Sick Kids on the day of the focus group).

**Participation:**
Participation in this research is voluntary. It is your/your child’s choice to take part in this study. You can stop at any time. The care your child gets at Sick Kids will not be affected in any way by whether you take part in this study.
New information that we get while we are doing this study may affect your decision to take part in this study. If this happens, we will tell you about this new information. And we will ask you again if you still want to be in the study.

If your child should become ill or are harmed because of study participation, we will treat your child for free. Your signing of this consent form does not interfere with your child’s legal rights in any way. The staff of the study and the hospital is still responsible legally and professionally, for what they do.

Sponsorship:
The study is sponsored by the Principal Investigator and Sick Kids.

Conflict of Interest:
I, and the other research team members have no conflict of interest to declare.

Consent:
By signing this form, I agree that:
1) You have explained this study to me. You have answered all my questions.
2) You have explained the possible harms and benefits (if any) of this study.
3) I know what I could do instead of taking part in this study. I understand that I have/my child has the right not to take part in the study and the right to stop at any time. My decision about taking part in the study will not affect my child’s health care at Sick Kids.
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7) I agree, or consent, to take part in this study.

Printed name of subject __________________________ Subject’s signature & date __________________________

Printed name of person who explained consent __________________________ Signature & date __________________________

Printed witness’s name (subject does not read English) __________________________ Witness’s signature & date __________________________

If you have any questions about this study, please call Megan Carricato at (416) 813-2119.

If you have questions about your rights as a subject in a study or injuries during a study, please call the Research Ethics Manager at (416) 813-5718.
Title of Research Project:
Quality of Life in Pediatric Patients with Intestinal Failure on Home Parenteral Nutrition

Investigators:
Paul Wales, MD General Surgery (416) 813-1490
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Vicky Ng, MD Transplant Centre (416) 813-7733

Purpose of the Research:
Quality of life is an important outcome measure of small bowel transplant. We would like to get a better understanding of your quality of life after your small bowel transplant. This study will help us learn about your overall physical functioning, psychological and social well-being and general well-being.

Description of the Research:
The study will ask you to fill out a questionnaire package that will contain questions about your physical functioning, psychological well-being and social activities. These questionnaires are meant to assess your quality of life. The study asks you to fill out a questionnaire package once. These questionnaires will be completed during the regularly scheduled clinic appointment and are expected to take approximately 45 minutes to complete.

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If you are asked to and agree to participate in a 1-hour focus group with other children, you will be required to complete an additional consent form.

Potential Harms:
We know of no harm that taking part in this study could cause you.

Potential Discomforts or Inconveniences:
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**Potential Benefits:**
This study will help to provide better care for some intestinal failure patients on home parenteral nutrition, as well as provide information about a comparison of quality of life pre and post small bowel transplant. This information is currently not available to professionals who care for these patients. This information will be used to improve the quality of the care provided to the intestinal failure population as a whole. Participants will receive a brief summary of the research findings.

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During the focus group meeting we will remind everyone that the information shared is private and should not be repeated outside the group, but we cannot be sure that information shared in that setting will be kept private. People in groups may share information with others outside the group.

**Reimbursement:**
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**Participation:**
Participation in this research is voluntary. It is your choice to take part in this study. You can stop at any time. The care you receive at Sick Kids will not be affected in any way by whether or not you take part in this study.
New information that we get while we are doing this study may affect your decision to take part in this study. If this happens, we will tell you about this new information and we will ask you again if you still want to be in the study.

If you become ill or are harmed because of study participation, we will treat you for free. Your signing of this consent form does not interfere with your legal rights in any way. The staff of the study, and the hospital are still responsible, legally and professionally, for what they do.

**Sponsorship:**
The study is sponsored by the Principal Investigator and Sick Kids.

**Conflict of Interest:**
None of the research team members appear to have a conflict of interest to declare.

**Consent:**
By signing this form, I agree that:
1) You have explained this study to me. You have answered all my questions.
2) You have explained the possible harms and benefits (if any) of this study.
3) I know what I could do instead of taking part in this study. I understand that I have the right not to take part in the study and the right to stop at any time. My decision about taking part in the study will not affect my health care at Sick Kids.
4) I am free now and in the future, to ask questions about the study.
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7) I agree to take part in this study.

____________________________________
Printed name of subject

Subject's signature & date

____________________________________
Printed name of person who explained consent

Signature & date

____________________________________
Printed witness's name (subject does not read English)

Witness's signature & date

If you have any questions about this study, please call Megan Carricato at (416) 813-2119.

If you have questions about your rights as a subject in a study or injuries during a study, please call the Research Ethics Manager at (416) 813-5718.

Page 3 of 3
ASSENT FORM*

Title of Study
Quality of Life in Pediatric Patients with Intestinal Failure on Home Parenteral Nutrition

Investigators:
Paul Wales, MD General Surgery (416) 813-1490
Megan Carricato, RD MSc(c) Transplant Centre (416) 813-2119
Stacey Pollock-BarZiv, PhD Transplant Centre (416) 813-3604
Julia Maxwell, MSW Department of Social Work (416) 813-6195
Nicole deSilva, CNS-NP Division of Neonatology (416) 813-6736
Vicky Ng, MD Transplant Centre (416) 813-7733

Why are we doing this study?
We would like to get a better understanding of your quality of life now that you have had a small bowel transplant. This study will help us learn about your health and well-being, about how you are doing overall, about school, about your friends and family, about everyday activities that you do, and how you feel in general. This information will help us understand what life is like for our patients after their small bowel transplant.

What will happen during the study?
You and/or your parent/guardian will be asked to fill out a questionnaire package one time during your regular follow-up clinic appointment. You may also be asked to participate in a 1 hour focus group.

Are there good things and bad things about the study?
This study may help us to make the care and services that we give to patients and families at Sick Kids better. There are no known harms from being in this study. The questionnaires might be a bit boring, but you will only need to fill them in once. The questionnaire package takes approximately 45 minutes to complete. If you have difficulty reading the questions or understanding the questions, someone will help you to go through them.

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Assent:
I was present when ____________________________ read this form and said that he or she agreed, or assented, to take part in this study.

__________________________  ____________________________
Printed name of person who obtained assent  Signature & Date
## Research Consent Form

*(Parent/Guardian of Participants)*

**Title of Research Project:**
Quality of Life in Pediatric Patients with Intestinal Failure on Home Parenteral Nutrition

**Investigators:**

<table>
<thead>
<tr>
<th>Name</th>
<th>Position</th>
<th>Contact Information</th>
</tr>
</thead>
<tbody>
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<td>Paul Wales, MD</td>
<td>General Surgery</td>
<td>(416) 813-1490</td>
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**Purpose of the Research:**

Quality of life is an important outcome measure of small bowel transplant. We would like to get a better understanding of your child’s quality of life now that they have had a small bowel transplant. This study will help us learn about your child’s overall physical functioning, psychological and social well-being and general well-being.

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A review of your child’s health record will be conducted to obtain important medical variables (such as age at home parenteral nutrition initiation, diagnosis, etc.)

**Potential Harms:**

We know of no harm that taking part in this study could cause your child.

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**Potential Benefits:**

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**Conflict of Interest:**
I, and the other research team members have no conflict of interest to declare.

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__________________________________________  __________________________
Printed name of subject                        Subject’s signature & date

__________________________________________  __________________________
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Printed witness’s name (subject does not read English)  Witness’s signature & date

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Appendix F

Focus Group Preparation

Verbal Introduction for Focus Groups

1. Parents/Primary Caregivers
Thank you all for coming today to our focus group. My name is Julia Maxwell and I am the social worker for the GIFT and small bowel transplant programs. I will be leading today’s focus group. My co-facilitators are Megan Carricato, a dietitian in the GIFT and liver, small bowel transplant programs as well as an investigator for the study. Nicole deSilva is a Certified Nurse Specialist – Nurse Practitioner in the Neonatal Intensive Care Unit here at The Hospital for Sick Children.

It is important that we understand what it is like to live at home with parenteral nutrition (or after a small bowel transplant). It is our hope and goal to improve the quality of life for patients who receive this therapy. We are going to ask you some questions about life at home for your child receiving the therapy, for yourselves and your families. Some questions are general in nature and others are more specific. It would be great if you can give us specific examples of your experiences. The conversations will be taped during the focus group, so if you do not want to be taped then we will miss this opportunity to information from you. We will assign each of you a colour and ask that you say this colour each time before you speak for transcribing the audiotapes to written documents afterwards.

The session will take approximately 2 hours of your time.

Now we will review the consent forms together. Please let us know at any time if you have questions or concerns.

Questions for Focus Groups

(A) PARENTS - HOME PARENTERAL NUTRITION FOCUS GROUP
1. What does quality of life mean to you?
2. What is YOUR perception on what your child’s quality of life is on home parenteral nutrition?
3. How does having a child on home parenteral nutrition impact the family?
4. When you think about home parenteral nutrition – what kinds of things have made your lives easier and what kinds of things make it harder?
5. Did you feel that the questionnaires addressed issues that you feel are related to quality of life?
Appendix G
Consent Forms for Qualitative Arm of Study
Title of Research Project:  
Quality of Life in Pediatric Patients with Intestinal Failure on Home Parenteral Nutrition

Principal Investigator:  
Paul Wales, MD  General Surgery  (416) 813-1490

Investigators:  
Megan Carricato, RD MSc(c)  Transplant Centre  (416) 813-2119
Stacey Pollock-BarZiv, PhD  Transplant Centre  (416) 813-3604
Julia Maxwell, MSW  Department of Social Work  (416) 813-6195
Nicole deSilva, CNS-NP  Division of Neonatology  (416) 813-6736
Vicky Ng, MD  Transplant Centre  (416) 813-7733

Confidentiality:  
The audio tapes produced from this focus group will be stored in a secure, locked location. Only members of the research team (and maybe the Sick Kids monitor) will have access to them. Following completion of the study the audio tapes will be kept as long as required in the Sick Kids Records Retention and Destruction policy. They will then be destroyed according to this same policy.

Consent:  
By signing this form,
1) I agree to be audio taped during this study. These audio tapes will be used to further understand the experience of having a child on home parenteral nutrition.
2) I understand that I have the right to refuse to take part in this study. I also have the right to withdraw from this part of the study at any time (e.g. before or after the audio tapes are made). My decision will not affect my/my child’s health care at SickKids.
3) I am free now, and in the future, to ask questions about the audio taping.
4) I have been told that my/my child’s medical records will be kept private. You will give no one information about me/my child, unless the law requires you to do so.
5) I understand that no information about me/my child (including these audio tapes) will be given to anyone or be published without first asking my permission.
6) I have read and understood pages 1 to 2 of this consent form. I agree, or consent, to being audio taped as part of the study focus group.

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<thead>
<tr>
<th>Printed name of subject</th>
<th>Subject’s signature &amp; date</th>
</tr>
</thead>
<tbody>
<tr>
<td>Printed name of person who explained consent</td>
<td>Signature &amp; date</td>
</tr>
<tr>
<td>Printed witness’s name (subject does not read English)</td>
<td>Witness’s signature &amp; date</td>
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## Appendix H
Quantitative Battery Assessment Administration

<table>
<thead>
<tr>
<th>Quantitative Tools</th>
<th>PROXY COMPLETION</th>
<th>SELF COMPLETION</th>
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<tbody>
<tr>
<td></td>
<td>2 months - 2 years</td>
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<td>Child Health Questionnaire Child Form (CHQ-CF87)</td>
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<td>PedsQL™ Parent Proxy Report (2-4 years)</td>
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<td>PedsQL™ Parent Proxy Report (5-7 years)</td>
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<td>PedsQL™ Parent Proxy Report (8-12 years)</td>
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<td>PedsQL™ Parent Proxy Report (13-18 years)</td>
<td></td>
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<tr>
<td>PedsQL™ Child Self Report (5-7 years)</td>
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<td>VAS I General Health</td>
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<td><strong>Total Scales for Completion Per Age Group</strong></td>
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<td><strong>7</strong></td>
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</table>
Appendix I
Visual Analogue Scales Proxy and Self Reports

PARENT: VISUAL ANALOGUE SCALES

1. Please place a mark across the line below that best describes how you feel your child’s overall “Quality of Life” has been these past 3 months:

Example:

[____________________________________]  
[____________________________________]
Best quality Best quality
of life of life

2. Please place a mark across the line below that best describes how much PAIN your child has have had these past 3 months:

Example:

[____________________________________]  
[____________________________________]
No Pain No Pain

3. Please place a mark across the line below that best describes how your child’s HEALTH IN GENERAL has been over these past 3 months:

Example:

[____________________________________]  
[____________________________________]
Best Overall Health Best Overall Health

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PARTICIPANTS: VISUAL ANALOGUE SCALES

1. Please place a mark across the line below that best describes how you feel your overall “Quality of Life” has been these past 3 months:

   Example:
   
   [______________________________]

   [______________________________]

   Best quality of life       Worst quality of life

2. Please place a mark across the line below that best describes how much PAIN you have had these past 3 months:

   Example:
   
   [______________________________]

   [______________________________]

   No Pain       Severe Pain

3. Please place a mark across the line below that best describes how your HEALTH IN GENERAL has been over these past 3 months:

   Example:
   
   [______________________________]

   [______________________________]

   Best Overall Health       Worst Overall Health
### Appendix J
Battery Assessment Summary of Results ᵇ Proxy Reports

<table>
<thead>
<tr>
<th>TOOL IN BATTERY ASSESSMENT PROXY RESPONSE</th>
<th>Home PN versus Norms</th>
<th>Home PN versus Published SBTx Sudan et al.</th>
<th>Home PN versus Published SBTx Ngo et al.</th>
<th>Home PN versus Institutional SBTx</th>
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<th>Home PN versus Norms</th>
<th>Home PN versus Published SBTx Sudan et al.</th>
<th>Home PN versus Published SBTx Ngo et al.</th>
<th>Home PN versus Institutional SBTx</th>
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Z: indicates statistically significant difference in scores at a p value <0.05 and a lower score in home PN sample
NS: non-significant difference in scores between comparison groups
n/a: scores not available for comparison
**Appendix K**

Battery Assessment Summary of Results ũ Self Reports

<table>
<thead>
<tr>
<th>TOOLS IN BATTERY ASSESSMENT SELF RESPONSE</th>
<th>Home PN versus Norms</th>
<th>Home PN versus Published SBTx</th>
<th>Sudan et al.</th>
<th>Home PN versus Institutional SBTx</th>
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<td>Behavior</td>
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<td>Family Cohesion</td>
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<tr>
<td>Global health</td>
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<td>Role/Social Limitations Owing to Behaviour</td>
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</tbody>
</table>

**PedSQL4.0™ Self Report Scales (5 to 18 years)**

| Physical Functioning Scale               | Z                    | n/a                           | NS          |
| Emotional Functioning Scale             | Z                    | n/a                           | NS          |
| Social Functioning                      | Z                    | n/a                           | NS          |
| School Functioning Scale                | Z                    | n/a                           | NS          |
| Psychosocial Summary Score              | Z                    | n/a                           | NS          |
| Total Score                             | Z                    | n/a                           | NS          |

**Visual Analogue Scales (8 to 18 years)**

| Quality of Life                          | n/a                  | n/a                           | n/a         |
| Pain                                     |                      |                               |             |
| Overall Health                           |                      |                               |             |

Z - indicates statistically significant difference in scores at a p value <0.05 and a lower score in home PN sample

NS - non-significant difference in scores between comparison groups

n/a - scores not available for comparison
# Appendix L

Results of PedsQL4.0™ Age-Specific Proxy Report Questionnaires for Home PN and Institutional SBTx

<table>
<thead>
<tr>
<th>PedsQL4.0™ Proxy-Report Scales</th>
<th>Home PN Sample Mean (Range)(^a)</th>
<th>Small Bowel Tx Sample Mean (Range)(^a)</th>
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<td>School Functioning Scale(^b)</td>
<td>70.8 (66.7 ÷ 75.0)</td>
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<td>Psychosocial Summary Score</td>
<td>63.3 (55.0 ÷ 73.1)</td>
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<td><strong>YOUNG CHILD (ages 5-7 yrs)</strong></td>
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<td>65.6 (50.0 ÷ 75.0)</td>
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<tr>
<td>Total Score</td>
<td>86.4 (84.8 ÷ 88.0)</td>
<td>58.7</td>
</tr>
<tr>
<td><strong>ADOLESCENT (ages 13-18 years)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Physical Functioning Scale</td>
<td>65.6 (53.1 ÷ 78.1)</td>
<td>-</td>
</tr>
<tr>
<td>Emotional Functioning Scale</td>
<td>62.5 (60.0 ÷ 65.0)</td>
<td>-</td>
</tr>
<tr>
<td>Social Functioning Scale</td>
<td>40.0 (10.0 ÷ 70.0)</td>
<td>-</td>
</tr>
<tr>
<td>School Functioning Scale</td>
<td>25.0 (20.0 ÷ 30.0)</td>
<td>-</td>
</tr>
<tr>
<td>Psychosocial Summary Score</td>
<td>42.5 (31.7 ÷ 53.3)</td>
<td>-</td>
</tr>
<tr>
<td>Total Score</td>
<td>50.5 (39.1 ÷ 62.0)</td>
<td>-</td>
</tr>
</tbody>
</table>

\(^a\)Ranges were provided where available, i.e. in samples where n>1 and when respondents did not achieve the same scale score
## Appendix M

Results of PedsQL4.0™ Age-Specific Self Report Questionnaires for Home PN and Institutional SBTx

<table>
<thead>
<tr>
<th>PedsQL4.0™ Self-Report Scales</th>
<th>Home PN Sample Mean (Range)(^a)</th>
<th>Small Bowel Tx Sample Mean (Range)(^a)</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>YOUNG CHILD (age 5-7 years)</strong></td>
<td></td>
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</tr>
<tr>
<td>Physical Functioning Scale</td>
<td>81.3</td>
<td>71.9 (68.8 – 75.0)</td>
</tr>
<tr>
<td>Emotional Functioning Scale</td>
<td>60.0</td>
<td>68.8 (50.0 – 87.5)</td>
</tr>
<tr>
<td>Social Functioning Scale</td>
<td>70.0</td>
<td>20.0 (0 – 40.0)</td>
</tr>
<tr>
<td>School Functioning Scale</td>
<td>70.0</td>
<td>31.3 (12.5 – 50.0)</td>
</tr>
<tr>
<td>Psychosocial Summary Score</td>
<td>66.7</td>
<td>38.7 (30.8 – 46.7)</td>
</tr>
<tr>
<td>Total Score</td>
<td>71.7</td>
<td>50.9 (45.2 – 56.5)</td>
</tr>
<tr>
<td><strong>CHILD (age 8-12 years)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Physical Functioning Scale</td>
<td>75.0 (65.6 – 84.4)</td>
<td>56.3</td>
</tr>
<tr>
<td>Emotional Functioning Scale</td>
<td>62.5 (55.0 – 70.0)</td>
<td>55.0</td>
</tr>
<tr>
<td>Social Functioning Scale</td>
<td>75.0 (60.0 – 90.0)</td>
<td>70.0</td>
</tr>
<tr>
<td>School Functioning Scale</td>
<td>75.0</td>
<td>20.0</td>
</tr>
<tr>
<td>Psychosocial Summary Score</td>
<td>70.8 (68.3 – 73.3)</td>
<td>48.3</td>
</tr>
<tr>
<td>Total Score</td>
<td>72.3 (67.4 – 77.2)</td>
<td>51.1</td>
</tr>
<tr>
<td><strong>ADOLESCENT (age 13-18 years)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Physical Functioning Scale</td>
<td>76.0 (62.5 – 84.4)</td>
<td>-</td>
</tr>
<tr>
<td>Emotional Functioning Scale</td>
<td>63.3 (45.0 – 80.0)</td>
<td>-</td>
</tr>
<tr>
<td>Social Functioning Scale</td>
<td>65.0 (10.0 – 95.0)</td>
<td>-</td>
</tr>
<tr>
<td>School Functioning Scale</td>
<td>43.3 (35.0 – 60.0)</td>
<td>-</td>
</tr>
<tr>
<td>Psychosocial Summary Score</td>
<td>57.2 (36.7 – 68.3)</td>
<td>-</td>
</tr>
<tr>
<td>Total Score</td>
<td>63.8 (45.6 – 72.8)</td>
<td>-</td>
</tr>
</tbody>
</table>

\(^a\) Ranges were provided where available, i.e. in samples where \(n>1\) and when respondents did not achieve the same scale score.
## Appendices N

Results of Questionnaire Assessment
(Missing and Redundant Answers, Ceiling and Floor Effects)

<table>
<thead>
<tr>
<th>Tool and Corresponding Scales (k=number of items per scale)</th>
<th>Number of Respondents (n)</th>
<th>Missing or Redundant Responses n (%)</th>
<th>Ceiling effects n (%)</th>
<th>Floor Effects n (%)</th>
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</thead>
<tbody>
<tr>
<td><strong>ITQOL</strong></td>
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<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Overall Health (k=1)</td>
<td>12</td>
<td>0</td>
<td>0</td>
<td>1 (8.3%)</td>
</tr>
<tr>
<td>Physical Abilities (k=10)</td>
<td>6</td>
<td>0</td>
<td>1 (16.7%)</td>
<td>0</td>
</tr>
<tr>
<td>Growth and development (k=10)</td>
<td>12</td>
<td>2 (1.7%)</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>Discomfort and pain (k=3)</td>
<td>12</td>
<td>0</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>Temperament and moods (k=18)</td>
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<td>0</td>
<td>0</td>
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<tr>
<td>Global Behavior (k=12)</td>
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<td>0</td>
<td>0</td>
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<tr>
<td>Getting along with others (k=15)</td>
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<td>0</td>
<td>0</td>
<td>0</td>
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<tr>
<td>General Health (k=11)</td>
<td>12</td>
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<td>0</td>
<td>0</td>
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<tr>
<td>Parent impact i emotional (k=7)</td>
<td>12</td>
<td>0</td>
<td>1 (8.3%)</td>
<td>0</td>
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<tr>
<td>Parent impact i time (k=7)</td>
<td>12</td>
<td>0</td>
<td>1 (8.3%)</td>
<td>0</td>
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<tr>
<td>Family Cohesion (k=1)</td>
<td>12</td>
<td>0</td>
<td>4 (33.3%)</td>
<td>0</td>
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<tr>
<td><strong>CHQ-PF50</strong></td>
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<tr>
<td>Physical Functioning (k=6)</td>
<td>9</td>
<td>0</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>Role/Social Limitations - Physical Function (k=2)</td>
<td>9</td>
<td>0</td>
<td>3 (33.3%)</td>
<td>1 (11.1%)</td>
</tr>
<tr>
<td>General Health (k=6)</td>
<td>9</td>
<td>0</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>Bodily Pain (k=2)</td>
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<td>0</td>
<td>3 (33.3%)</td>
<td>0</td>
</tr>
<tr>
<td>Role/Social Limitations - Emotions/Behavior (k=3)</td>
<td>9</td>
<td>0</td>
<td>3 (33.3%)</td>
<td>2 (22.2%)</td>
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<tr>
<td>Self Esteem (k=6)</td>
<td>9</td>
<td>2 (3.7%)</td>
<td>0</td>
<td>0</td>
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<tr>
<td>Mental Health (k=5)</td>
<td>9</td>
<td>0</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>Behavior (k=6)</td>
<td>9</td>
<td>0</td>
<td>0</td>
<td>0</td>
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<tr>
<td>Family Activities (k=6)</td>
<td>9</td>
<td>0</td>
<td>0</td>
<td>1 (11.1%)</td>
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<tr>
<td>Family Cohesion (k=1)</td>
<td>9</td>
<td>1 (11.1%)</td>
<td>2 (22.2%)</td>
<td>0</td>
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<tr>
<td>Parental Impact on Emotion (k=3)</td>
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<tr>
<td>Parental Impact on Time (k=3)</td>
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<td>2 (22.2%)</td>
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<td><strong>CHQ-CF87</strong></td>
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<tr>
<td>Physical Functioning (k=9)</td>
<td>4</td>
<td>0</td>
<td>2 (50%)</td>
<td>0</td>
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<tr>
<td>Role/Social Limitations - Physical Function (k=3)</td>
<td>4</td>
<td>0</td>
<td>1 (25%)</td>
<td>0</td>
</tr>
<tr>
<td>General Health (k=12)</td>
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<td>Bodily Pain (k=2)</td>
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<td>0</td>
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<td>Role/Social Limitations - Emotions (k=3)</td>
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<td>0</td>
<td>2 (50%)</td>
<td>0</td>
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<td>Self Esteem (k=14)</td>
<td>4</td>
<td>1 (1.8%)</td>
<td>0</td>
<td>0</td>
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<td>Mental Health (k=16)</td>
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<tr>
<td>Behavior (k=17)</td>
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<td>Family Activities (k=6)</td>
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<td>Family Cohesion (k=1)</td>
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<tr>
<td>Role/Social Limitations ⊤ Behavior (k=3)</td>
<td>4</td>
<td>0</td>
<td>4 (100%)</td>
<td>0</td>
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Continued on next page
<table>
<thead>
<tr>
<th>Tool and Corresponding Scales (k=number of items per scale)</th>
<th>Number of Respondents (n)</th>
<th>Missing or Redundant Responses n (%)</th>
<th>Ceiling effects n (%)</th>
<th>Floor Effects n (%)</th>
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<tr>
<td>PedsQL™ (Self – All Ages)</td>
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<tr>
<td>Physical Functioning (k=8)</td>
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<td>0</td>
<td>0</td>
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<tr>
<td>Emotional Functioning (k=5)</td>
<td>9</td>
<td>1 redundant (2.2%)</td>
<td>0</td>
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<td>Social Functioning (k=5)</td>
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<td>0</td>
<td>1 (2.2%)</td>
<td>0</td>
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<td>School Functioning (k=5)</td>
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<td>1 redundant (2.2%)</td>
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<td>PedsQL™ (Proxy – All Ages)</td>
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<tr>
<td>Emotional Functioning (k=5)</td>
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<td>Social Functioning (k=5)</td>
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<td>1 (4.8%)</td>
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<td>Emotional Functioning Scale (k=5)</td>
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<td>1 (4.8%)</td>
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<td>Social Functioning (k=4)</td>
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<td>Worry (k=5)</td>
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<td>Daily Activities (k=3)</td>
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<td>Family Relationships (k=5)</td>
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<td>1 (4.8%)</td>
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<td>Visual Analogue Scale – Self</td>
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<tr>
<td>Quality of Life</td>
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<td>1 (16.7%)</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>Pain</td>
<td>6</td>
<td>1 (16.7%)</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>Overall Health</td>
<td>6</td>
<td>1 (16.7%)</td>
<td>0</td>
<td>0</td>
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<tr>
<td>Visual Analogue Scale – Proxy</td>
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<tr>
<td>Quality of Life</td>
<td>21</td>
<td>2 (9.5%)</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>Pain</td>
<td>21</td>
<td>0</td>
<td>0</td>
<td>0</td>
</tr>
<tr>
<td>Overall Health</td>
<td>21</td>
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<td>All Items (k=14)</td>
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<td>3 (14.3%)</td>
<td>2 (9.5%)</td>
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