A PILOT STUDY ON PEDSQL™ MODUL IMPAK KELUARGA AMONG CAREGIVERS OF CHILDREN WITH TRANSFUSION DEPENDANT THALASSAEMIA IN HOSPITAL USM, MALAYSIA

DR SURIA EMILIA SUHANA BINTI OTHMAN TAN

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LIST OF ABBREVIATIONS

TDT	: Transfusion dependant thalassaemia
PEDSQL™	: Pediatric quality of life inventory™
HRQOL	: Health related quality of life
Hospital USM	: Hospital Universiti Sains Malaysia
MOH	: Ministry of Health
SLR	: Simple linear regression
SD	: Standard deviation
CI	: Confidence interval
PASW	: Statistical Predictive Analytics Software
MRKH syndrome	: Mayer-Rokitansky-Küster-Hauser syndrome
RM	: Malaysian ringgit

DEFINITIONS

Transfusion dependant thalassaemia:

Transfusion dependant thalassaemia patients are those who require life-long regular blood transfusions (Clinical Practice Guidelines: Management of transfusion dependant thalassaemia, November 2009)

In this study, a transfusion dependant thalassaemia patient refers to thalassaemia patient on blood transfusions of at least eight weeks interval (Dahlui *et al.*, 2009)

Health-related Quality of Life (HRQOL):

Subjective and multidimensional, encompassing physical and occupational function, psychological state, social interaction and somatic sensation (Carol Estwing Ph.D. and Ferrans, 2004).

Subjective and objective impact of dysfunction associated with an illness or injury, medical treatment, and health care policy (Spieth and Harris, 1996)

ABSTRAK

Latar belakang:

Talasemia adalah salah satu penyakit gangguan darah genetik yang paling kerap di dunia. Ia dicirikan oleh kekurangan sintesis rantai globin, yang membawa kepada erythropoiesis tidak berkesan, anemia teruk dan penyakit kronik Di kalangan kanak-kanak dan orang dewasa. Pesakit talasemia kanak-kanak memerlukan pemindahan darah secara berkala sepanjang hayat untuk memanjangkan hayat hidup dan mengurangkan penghasilan darah di luar sum-sum tulang. Seperti mana-mana penyakit kronik lain, talasemia bukan sahaja memberi kesan kepada pesakit sebagai individu, ia juga melibatkan pelbagai beban emosi dan psikologi kepada ibu bapa dan keluarga secara keseluruhan. Malangnya, hanya beberapa kajian yang pernah dijalankan di seluruh dunia untuk menilai kualiti hidup ibu bapa kanak-kanak yang bergantung kepada pemindahan darah berkala yang merupakan sebab mengapa kajian ini adalah penting untuk dijalankan.

Objektif:

Untuk mengkaji kualiti hidup ibu bapa dan fungsi keluarga pesakit talasemia yang memerlukan rawatan pemindahan darah berkala di Hospital Universiti Sains Malaysia.

Kaedah:

Kajian rintis telah dijalankan di kalangan 31 ibu bapa kanak –kanak yang memerlukan pemindahan darah berkala yang menghadiri klinik pediatrik atau dimasukkan ke wad untuk pemindahan darah di Hospital USM dari 1 Oktober 2012 hingga 1 Mei 2013. PedsQL^{TM} Modul Impak Keluarga telah digunakan. Analisis univariable (regresi linear mudah dan Mann Whitney Test) telah digunakan untuk membandingkan tahap kualiti hidup ibu bapa dan fungsi keluarga antara data kanak-kanak dan data penjaga.

Keputusan:

Markah purata untuk jumlah keseluruhan impak ialah 75.73 (SD: 14.42), markah purata untuk kualiti hidup ibu bapa ialah 74.05 (SD: 14.65) dan markah purata bagi fungsi keluarga ialah 81.65 (SD: 17.21). Pendapatan keluarga didapati menjadi faktor penting untuk markah jumlah keseluruhan impak dan markah kualiti hidup ibu bapa, dengan nilai *b* 0.002 (95% CI: 0.000, 0.004) bagi kedua-dua markah. Penjaga lelaki, penjaga dengan tahap pendidikan lebih tinggi dan penjaga kanak-kanak yang tidak mempunyai penyakit yang berkaitan mencatatkan markah jumlah keseluruhan impak yang lebih tinggi.

Kesimpulan:

Kualiti hidup ibu bapa dan fungsi keluarga di kalangan penjaga kanak-kanak talasemia yang memerlukan pemindahan darah berkala dalam kajian ini adalah agak memuaskan. Pendapatan keluarga merupakan faktor penting yang mempengaruhi kualiti hidup ibu bapa dan fungsi keluarga.

ABSTRACT

Background:

Thalassaemia is one of the commonest genetic blood disorders in the world. It is characterized by inadequate globin chain synthesis, leading to ineffective erythropoiesis, severe anaemia and chronic disease in children and adults. A child with thalassaemia requires lifelong regular blood transfusion in order to prolong survival and reduce extramedullary haematopoiesis. As any other chronic illness, thalassaemia does not only affect patient as an individual; it also involves variety of emotional and psychological burden to the parents and family as a whole. Unfortunately, there are very few studies done worldwide to assess parental heelth related quality of life (HRQOL) in transfusion dependant thalassaemia which is the reason why this study is important.

Objectives:

To study the parental health related quality of life and family functioning among children with transfusion dependant thalassaemia in Hospital Universiti Sains Malaysia and its associated factors.

Methodology:

A pilot study was conducted among 31 caregivers of transfusion dependant thalassaemia children attending paediatric clinic or admitted for blood transfusion in Hospital USM from 1st October 2012 to 1st May 2013. PEDSQL™ "Modul Impak Keluarga" was used. Univariable analysis (simple linear regression and Mann Whitney Test) was used to compare the level of parental HRQOL and family function between children and caregivers profile.

Results:

The mean total impact score was 75.73 (SD: 14.42), mean score for parental HRQOL was 74.05 (SD: 14.65) and the mean score for family function was 81.65 (SD: 17.21). Family income was found to be the significant factor for total impact score and parental HRQOL score, with *b* of 0.002 (95% CI: 0.000 - 0.004, $p < 0.04$) for both of the score. Male caregivers ($p < 0.04$), caregivers with higher educational level ($p < 0.02$) and caregivers of children with no associated illness ($p < 0.045$) had significantly higher total impact score.

Conclusion:

The parent's quality of life and family functioning among caregivers of children with transfusion dependant thalassaemia in this study was generally satisfactory but family income was an important factor affecting parental HRQOL and family functioning in children with transfusion dependant thalassaemia.

CHAPTER 1

INTRODUCTION

Thalassaemia is one of the commonest genetic blood disorders worldwide (Cao *et al.*, 2007). In Malaysia as well as many other countries, thalassaemia also poses an important public health concern (Wong *et al.*, 2011). It is more common in Malays and Chinese compared to the Indian and other ethnics groups (George *et al.*, 2001). Approximately 240 million people are heterozygous for β-thalassaemia and estimated 200,000 affected homozygous are born each year(Cao *et al.*, 2007). In Malaysia, it was estimated that around 150 to 350 babies were born with thalassaemia each year (Ismail *et al.*, 2006).

A child with beta thalassemia usually is well at birth, but they will develop progressive anemia due to partial or total absence in hemoglobin, which will lead to early death without blood transfusion. In those who survive, the condition imposes serious implications on their health and quality of life as well as their families.

Parents of children with transfusion dependant thalassaemia need to accompany their child for monthly blood transfusion in which they usually spend the whole day in the

hospital. The children need to come in the morning for blood investigations to check for hemoglobin level and blood cross match and then, they need to wait for the blood products to be available. Usually, this process takes about two to three hours but it may be prolonged if there is a problem with the children's blood. For example, if the children develop autoantibodies towards the red blood cell, the blood bank needs to do further test to find the most suitable blood for the child. It may take another one or two days depending on the availability of blood and severity of the autoantibodies. Thus, the parents need to bring the child home first and then bring back the child to the hospital once the blood is ready. For the uncomplicated situation, the children need to wait for two to three hours for blood cross matching and then only the blood transfusion can be started. The duration for each transfusion usually takes about 4 hours.

The children also need frequent iron chelation therapy in order to reduce the impact of transfusion-related toxicity due to iron overload. This iron chelation therapy is available in subcutaneous and oral form. Unfortunately, this therapy also has its own adverse effects and limitations that contribute to noncompliance to the drugs. Patient who does not comply to iron chelation therapy often develop various complications such as cardiomyopathy, endocrinopathy, infection, coagulopathy, liver toxicity and others that might compromising the child and family quality of life further. Currently, only successful hematopoietic stem cell transplant (HSCT) can offer a permanent cure but it is limited by HLA-matched sibling donor and procedure related complication (Cheuk *et al.*, 2008). Thus, parents of these children are exposed to protracted physical and

emotional suffering because of their children's devastating health problem leading to disruption of their normal psychosocial life.

Previous literature has mainly focused on assessing health-related quality of life in children with beta thalassaemia but less is known about their parents and families (Ismail *et al.*, 2006; Dahlui *et al.*, 2009; Clarke *et al.*, 2010). Findings of studies conducted elsewhere might not be the reflection of true Malaysian due to difference in their ethnicity, religion and socioeconomic background of the family. Therefore, this study was conducted to look at the impact of having a child with beta thalassaemia on the parents' health-related quality of life (HRQOL) and family functioning in Kelantan.

CHAPTER 2

LITERATURE REVIEW

Chronic illness in childhood has a substantial impact on the patient's life and on the whole family system. A paediatric illness is defined as a chronic health problem if it lasts over twelve months, affects the child's normal activities, and requires a lot of hospitalizations and/or home health care and/or extensive medical care (Stein and Silver, 1999). Examples of chronic paediatric illness are cerebral palsy, haemoglobinopathies, cancer, heart disease, diabetes mellitus, epilepsy, bronchial asthma and many others.

Thalassaemia is a group of inherited haematologic disorders caused by defects in the synthesis of one or more of the hemoglobin chains. Alpha thalassaemia is caused by reduced or absent synthesis of alpha globin chains, and beta thalassaemia is caused by reduced or absent synthesis of beta globin chains. Imbalances of globin chains cause haemolysis and impair erythropoiesis. Silent carriers of alpha thalassaemia and those with alpha or beta thalassaemia trait are asymptomatic and require no treatment. Alpha thalassaemia intermedia or haemoglobin H disease cause haemolytic anemia. Alpha thalassaemia major with haemoglobin Bart's usually results in fatal hydrops foetalis. Beta thalassaemia major causes haemolytic anemia, poor growth, and skeletal abnormalities during infancy. Affected children will require regular lifelong blood

transfusions. Clinical manifestations of beta thalassaemia intermedia patients are less severe than beta thalassaemia major patient and might require only episodic blood transfusions. Transfusion dependant patients will develop iron overload from repeated blood transfusion and require iron chelation therapy to remove the excess iron. Alternative therapy for beta thalassaemia major is bone marrow transplant which could be curative for some children especially if recipient is compatible with donor (Muncie and Campbell, 2009).

Haemoglobin consists of an iron-containing heme ring and four globin chains: two alpha and two non alpha. The composition of the four globin chains determines the haemoglobin type. Fetal haemoglobin (HbF) has two alpha and two gamma chains (alpha2 gamma2). Adult haemoglobin A (HbA) has two alpha and two beta chains (alpha2 beta2), whereas haemoglobin A2 (HbA2) has two alpha and two delta chains (alpha2 delta2). At birth, HbF accounts for approximately 80 percent of haemoglobin and HbA accounts for 20 percent of hemoglobin (Richardson, 2007). The transition from gamma globin synthesis (HbF) to beta globin synthesis (HbA) begins before birth. By approximately six months of age, healthy infants will have transitioned to mostly HbA, a small amount of HbA2, and negligible HbF (Muncie and Campbell, 2009).

Beta thalassaemia is the result of deficient or absent synthesis of beta globin chains, leading to excess alpha chains. Beta globin synthesis is controlled by one gene on each chromosome 11. Beta thalassaemia occurs from any of more than 200 point mutations and (rarely) deletions of the two genes (Olivieri, 1999). Beta globin chain production can range from near normal to completely absent, leading to varying degrees of excess

alpha globin to beta globin chain production hence varying degree of clinical presentation or severity. The one gene defect, beta thalassaemia trait (minor), is asymptomatic and results in microcytosis and mild anemia. If the synthesis from both genes is severely reduced or absent, the person has beta thalassaemia major. Persons with beta thalassaemia major are almost never symptomatic at birth because of the presence of HbF, but symptoms begin to develop by six months of age. If the synthesis of beta chains is less severely reduced, the person has beta thalassaemia intermedia. These persons experience symptoms that are less severe and do not require lifelong transfusions (Olivieri, 1999).

Transfusion dependant thalassaemia as other chronic pediatric illness does not only affect patient as an individual; it also involves variety of emotional and psychological burden to the parents and family as a whole. The impact to the family varies depending on the severity and duration of the illness as well as coping abilities of the affected family. There are very few studies done worldwide to assess parental HRQOL in transfusion dependant thalassaemia. Hence, the outcome of studies looking at parental HRQOL in other chronic disorders are also reviewed here for comparison. One example of other chronic disease is congenital heart disease. Arafa (Arafa *et al.*, 2008) measured the quality of life among parents of children with heart disease as compared to parents of children with minor diseases. This comparative cross sectional study involved 400 patients with heart disease (congenital or rheumatic heart disease) and equal number of parents accompanying their children who suffered from minor illness (upper respiratory tract infection, sore throat, abscess and diarrheal disease). A structured questionnaire

were used to elicit information regarding their socioeconomic, heart disease related data and family related risk data. Parents of children with heart disease were found to have significantly poorer quality of life compared to parents of children with minor illness. They were few factors found to have an impact to the quality of life. It includes age of the affected child, number of children in the family, financial aspect of the family and presence of other comorbid conditions. The study concluded that with a significantly poor quality of life in the family with heart disease, treatment decisions must take into account of psychological status, social support and reassurance of the parents involved.

Another study looking at the quality of life among patients, parents and medical providers in children and adolescents with congenital and acquired heart disease revealed a different findings (Marino *et al.*, 2009). This study involved patients with heart disease ranging from ages eight to eighteen years old as well as their parents and healthcare providers that directly involved in the management of these patients. The parameter of the study involved physical, psychological, social, school and others. The researchers concluded that physical limitations were the most affected parameters in the patient and parent.

Cerebral palsy is another example of common chronic condition in children. Raina (Raina *et al.*, 2005) carried a study involving 468 families of children with cerebral palsy in Ontario, Canada. Since cerebral palsy patients did not only suffer from impaired motor function but with other associated disabilities such as sensory, intellectual and

communicative impairment, the researchers developed a stress model and applied structural equation model to study the impact of cerebral palsy children to their family. Their methodology involved variables such as child's health, behavior and functional status, parental characteristics, social supports, family function and parents' health and psychological status. The researchers found that the most important predictors of caregivers well being were the child behavior, care giving demands and family function. Higher level of behavioral problems in children were associated with lower levels of both psychological and physical health of the caregivers, where as fewer child behavior problems were associated with higher self perception and a greater ability to manage stress. The stress management and self perception were predictors to good psychological health among the caregivers. Researchers concluded that the psychological and physical health of caregivers, in particular mothers, were strongly influenced by child behavior and care giving demands, and thus supports management pathways that were more biopsychosocial in nature and more family-centric.

Multiple literatures had been published in regards to other chronic haematological pediatric illness such as sickle cell disease. Panepinto *et al.* (Panepinto *et al.*, 2009) in Hospital of Wisconsin/Medical College of Winconsin conducted a cross sectional study involving 170 parents of children with sickle cell disease and parents without children with sickle cell disease. The researchers applied psychometric evaluation of the parents using Family Impact Module. The children were assessed whether they had comorbid medical conditions such as neurological or chest symptoms, behavioural comorbidities such as attention or anxiety problems and degree of severity of their sickle cell disease.

The participants were further divided into five subsets namely sickle cell without comorbidities, sickle cell with neurobehavioural comorbidities, sickle cell with medical comorbidities, sickle cell disease with both neurobehavioural and medical comorbidities and healthy control groups. The researchers found that parents of children with sickle cell disease demonstrated more worry with communications problems in their children compared with parents of normal healthy children. However, the Family Impact Module in these study did not demonstrate differences between the two groups of parents with regards to family function and parents' quality of life.

Impact of newly diagnosed chronic pediatric illness on parental quality of life have also been studied and published. Goldbeck (Goldbeck, 2006) from University Clinic Ulm, Germany conducted a research to study parental functioning and well being in adapting to chronic pediatric conditions. He investigated the effect of diagnosis in cancer, diabetic and epileptic patients and time of diagnosis to parents' quality of life. Cancer, diabetes mellitus and epilepsy are three distinct chronic illnesses that present with different implications to the affected family. Even though there were multiple studies in regards to the effects of chronic pediatric illness to parents and family, the researcher noted that none have studied with respect to different types of chronic illnesses as above. In his study, he hypothesized that the problems related to cancer were more towards elevated parental distress due to life threatening nature of the illness, compared with diabetes mellitus and epilepsy which involves more compliance and surveillance issues. Hundred twenty-two parents of patients with one of the mentioned chronic illness were required to fill in questionnaires during 1-2 week and 2-3 months after diagnosis.

Parents' quality of life was then analyzed. The study concluded that parents of children with cancer showed significantly lower parental quality of life compared to parents of children with diabetes mellitus and epilepsy thus indicates that parents of newly diagnosed cancer patients need more psychosocial support in time.

With regards to beta thalassaemia, there were many published literature regarding quality of life of affected children but only few studied the quality of life of the caregivers. In a study titled 'Health-related quality of life and financial impact of caring for a child with thalassaemia major in the UK', Clarke (Clarke *et al.*, 2010) conducted a cross sectional study of health related quality of life in 22 mothers of Thalassemia Major aged 8-18 who came for treatment at three UK Pediatric Hematology and Bone Marrow Transplant centers. Researchers use Pediatric quality of life (PedsQL 4.0) questionnaire which comprised of 23 items with assessment ranges from physical, social, emotional and school function. Other tools used in this study were Strengths and Difficulties Questionnaire, SDQ that assessed child emotional and behavioural problems. Even though the results did not show significant behavioural and emotional changes in children with thalassaemia major, there were reduction in overall health related quality of life, despite free treatment and allowance provided by National Health Services in the UK. These findings could be due to the relatively small sample size and reliance on mothers' report. The author recommended that social workers need to be more proactive to fulfill the needs of patients and families of thalassaemia major.

In Iran, few studies related to thalassaemia and health related quality of life had been published, notably by Baghianimoghadam and Sharghi. Baghianimoghadam (Baghianimoghadam *et al.*, 2011) performed a cross sectional study involving 60 thalassaemia patients and 120 healthy children as control group at Shahid Sadoughi University of Medical Sciences, Yazd, Iran. The study used self administered short form questionnaire of Medical Outcomes study (SF-20) that measures functional (physical, social and role) and well being (mental health, health perception and pain). Baghianimoghadam found that patients with thalassaemia showed reduction in health related quality of life compared to healthy control group by 14% to 23%. He also noted that the findings of the study correspond to another study by Adriana Ismail (Ismail *et al.*, 2006) in Malaysia, showing reduction in health related quality of life in all domains (physical, social and mental activities) of thalassaemia patient.

Ismail (2006) did a cross sectional study using PEDSQL^{TM} 4.0 questionnaire among transfusion dependant thalassaemia patients in Hospital Kuala Lumpur. The questionnaire were divided into several subscales which includes physical, emotional, social and school functioning and it was compared with healthy group. The study involved 78 thalassaemia patients and 235 healthy controls that agreed to participate. The result showed significantly lower health related quality of life score in thalassaemia group compared to healthy group especially in the physical, social and school function domain by 10 to 24 percent. However, there were no significant difference noted with regard to gender, ethnicity and household income among thalassaemia patients and

healthy children. The limitation of the study was that it was conducted in a single center, thus might not be representative of populations of the whole country.

Sharghi (Sharghi *et al.*, 2006) studied depressive mood disorders among mothers of children with thalassaemia and other blood cancers. This study put particular focus on mothers, due to increased responsibilities of mothers on taking extra care of their children with thalassaemia and other blood cancers. This is important in order to identify mothers with increased psychological risks of developing depression. The cross sectional study was performed at Ardebil University of Medical Sciences, Iran. It involved 294 mothers that were divided into three groups (thalassaemia, blood cancer and healthy control). The mothers were assessed using Beck Depression Inventory, a self administered test using objective method in measuring intensity of depressive symptoms, in which mothers with test scores equal or higher than 19 were considered depressed. The authors noted that mothers within the thalassaemia and blood cancer groups showed higher prevalence to depression than the control group.

PEDSQL™ Family Impact Module was originally developed by James Varni (Varni *et al.*, 2004) to measure the impact of pediatric chronic health conditions on the parent health related quality of life and family functioning. It measures parents' self reported physical, emotional, social and cognitive functions, communications, worry, parent-reported family daily activities and family relationship. The original version of the PEDSQL™ Family Impact Module has been validated by the University of California,

San Diego, California, USA involving 23 families of medically fragile children with complex health condition who either resided in long term care hospital or resided at home with their families. The study demonstrated good internal consistency with the Cronbach's alpha score ranges from 0.82 to 0.97.

The PEDSQL^{TM} Family Impact Module has been widely translated into different languages such as Portuguese, Chinese and Malay. It has also been validated and tested to caregivers of children with several pediatric chronic conditions such as sickle cell disease (Panepinto *et al.*, 2009), children with disabilities (Rahman *et al.*, 2011), pediatric asthma and heart disease (Chen *et al.*, 2011), children with chronic pain (Jastrowski Mano *et al.*, 2011) and pediatric cancer (Scarpelli *et al.*, 2008). All of the above studies had demonstrated strong internal consistency (Cronbach's alpha score >0.7).

The Chinese version of PEDSQL^{TM} Family Impact Module was validated by Chen (Chen *et al.*, 2011) from Sun Yat-sen University, Guangzhou, China. It was a cross sectioned study to measure family impact on children with both asthma and heart disease. The questionnaire was administered to 136 parents of children with asthma and 264 parents of children with heart disease from four tertiary hospitals. The results showed than families with asthmatic children had higher health related quality of life compared to parents of children with heart disease and concluded that the Chinese

version of PEDSQL™ Family Impact Module had adequate psychometric properties to be used to parents of children with chronic illnesses such as asthma.

The Brazilian Portuguese version of PEDSQL™ Family Impact Module had been used successfully in a study by Scarpelli (Scarpelli *et al.*, 2008). The family impact module was translated in Brazilian Portuguese and administered to 95 parents of children with pediatric cancer at two public hospitals. The results showed that construct validity of the module and reliability of the module ranges from regular to nearly perfect. The authors concluded that the Brazilian Portuguese version of PEDSQL™ Family Impact Module demonstrated adequate properties with regards to validity and reliability of the construct.

The Malay version of PEDSQL™ Family Impact Module was translated by Rahman (Rahman *et al.*, 2011) and validated among children with disabilities in Kelantan. The internal reliability of consistency was good with Cronbach's alpha score >0.7 for all domains studied and the authors concluded that the reliability and validity of the construct were adequate.

The impact of children with chronic illnesses, such as heart disease, sickle cell disease, cancer and child with disabilities on parents and families has been shown to be significant by many researchers. However, there are only few literatures assessing the impact of thalassaemia to the parents and family and none of them used PEDSQL™

Family Impact Module to assess parent HRQOL and family functioning in thalassaemia. As thalassaemia is highly prevalent in Malaysia, it is important for pediatrician to understand the impact of the illness to the parents and the family functions. It is equally important to indentify the factors that could contribute to poor quality of life.

CHAPTER 3

OBJECTIVES

3.1 General Objective:

To study the parental health related quality of life and family functioning among children with transfusion dependant thalassaemia in Hospital Universiti Sains Malaysia and its associated factors.

3.2 Specific Objectives:

1. To determine the parent and family functioning of children with transfusion dependant thalassaemia in Hospital USM using PEDSQL^{TM} Modul Impak Keluarga.
2. To compare the level of parental HRQOL and family function between caregivers profile and children's profile.

3.3 Study Hypotheses:

1. The parental HRQOL and family functioning among caregivers of children with thalassaemia are similar compared to other chronic illness.
2. The parental HRQOL and family functioning are similar between caregivers profile and children profile.

CHAPTER 4

METHODOLOGY

4.1 Study design

This is a pilot study that was conducted in Paediatric wards (6U and 6S), thalassaemia daycare centre and paediatric clinic Hospital Universiti Sains Malaysia from 1st October 2012 till 1st May 2013.

4.2 Reference population

All caretakers of transfusion dependant thalassaemia children receiving treatment in Hospital Universiti Sains Malaysia.

4.3 The source population

All caretakers accompanying their children with transfusion dependant thalassaemia in paediatric wards, daycare centre and paediatric clinic in Hospital Universiti Sains Malaysia within 1st October 2012 to 1st May 2013.

4.4 Inclusion and exclusion criterias

4.4.1 Inclusion criterias

- All caretakers of children with transfusion dependant thalassaemia who are available during period of data collection and must be able to read Malay language.

- All caretakers with transfusion dependant thalassaemia between ages 6 month to 18 years old.

4.4.2 Exclusion criterias

- Caretakers of children with non transfusion dependant thalassaemia.

- Caretakers of children with transfusion dependant thalassaemia who do not give consent for the study.

- Caretakers of children with transfusion dependant thalassaemia who do not understand Malay language.

- Caretakers of children with transfusion dependant thalassaemia age less than 6 month or more than 19 years old.

4.5 Sampling frame

Caretakers who fulfilled the inclusion criteria.

4.6 Sample size determination

As this was a pilot study, sample size was obtained as a guide for estimating sample size that we could used. For first objective, sample size was determined using single mean formula:

$$n : \left\{ \frac{z\sigma}{\Delta} \right\}^2$$

where; z : the value to estimate the 95% confidence interval (1.96)

σ: standard deviation comes from previous study

Δ (precision): the difference between expected mean in study and the true mean

For **first objective**, the calculation done is shown in the Table 4.1:

Table 4.1: Sample Size Calculation for First Objective

OBJECTIVE	σ	n + 10% NON RESPONDING SAMPLE
1a) **parental HRQOL summary Score**	σ : 15.5 (Varni *et al.*, 2004)	Calculation: $\left\{\frac{1.96 \times 15.55}{5}\right\}^2$ n : 36 (+10%) : 39
1b) **Family summary score**	σ : 20.47 (Varni *et al.*, 2004)	Calculation: $\left\{\frac{1.96 \times 20.47}{5}\right\}^2$ n : 64 (+10%) : 70

For **parent functioning**, the sample size calculated (using SD: 15.55) after adding 10% of non response is **39 samples**.

For **family functioning**, the sample size calculated (using SD: 20.47) after adding 10% of non response is **70 samples**.

For **second objectives**, the sample size was calculated using PS software (for family income)

Alpha (α) was set at **0.05**

Power of study (1 – β) : 0.8

σ is a **standard deviation (SD)** from previous study; that is the SD of total impact family score among gender of caregivers.

In this study, SD was estimated from SD of mean total family impact score among all caregivers : **17.06** (Varni *et al.*, 2004)

δ (detectable difference in means) : 15

The smallest but clinically meaningful difference in the mean of total impact family score between caregivers gender.

m: ratio between the prevalence of transfusion dependant thalassaemia in male and female caregivers taken to be **1** in Kelantan population.

Sample size determined after adding 10% of non response rate was 23 for each group, ie total sample of 46.

Sample size chosen for the study

The sample size chosen for the study was 46 respondents, the sample size calculated for objective 2 (the smallest sample size). This is due to short duration of study and limited number of transfusion dependant thalassaemia attending treatment in Hospital USM. However, the final number of sample size in this study is 31 children.

4.7 Sampling method

Non probability sampling method was applied. All eligible caretakers were taken.

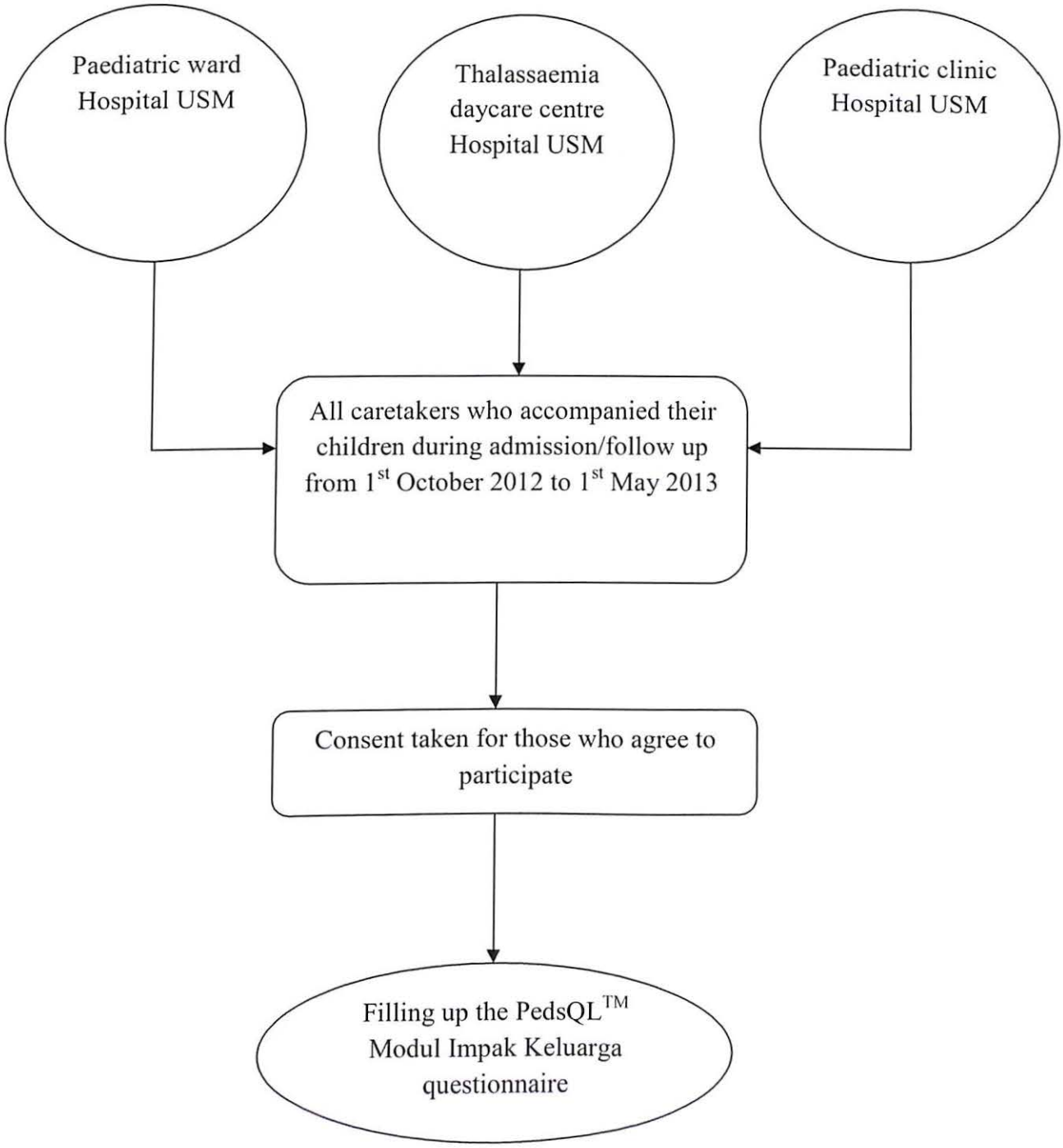

Figure 1.1: A schematic diagram of selection of eligible caretakers.