TRANSITION TOGETHER: A STUDY OF PEDIATRIC PATIENTS WITH SICKLE CELL DISEASE AS THEY TRANSITION TO ADULT HEALTH CARE

by

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ABSTRACT

Introduction: Improvement in medical care for sickle cell patients, translating into improvement in survival, has created the need for an active and intentional process of transition from pediatric to adult oriented health care.

Objective: The purpose of this study was to determine the perspectives of adolescents and young adults with sickle cell disease as they prepare to transition, and to evaluate their current health care knowledge base and skills.

Methods: A sample of 18 pediatric sickle cell patients age 15-22 years were recruited from a comprehensive Sickle Cell Center. Two sections of a four part questionnaire containing open- and closed-ended questions were assessed in order to explore these patients' expectations and concerns about the transition process, and to evaluate their self-management of sickle cell disease.

Results: Themes identified from the open-ended questions include: 1) Apprehension to Leaving Pediatric Care, 2) Forming New Relationships with Adult Providers, 3) Growing Up and Responsibility, 4) Worries Regarding Readiness and Support, and 5) Active Role in Transition. Statistical analysis of the close-ended questions revealed that patients appeared to have similar levels of independence in performing skills related to "Knowledge of Health

Issues/Diagnosis, “Being Prepared”, and “Taking Charge”. However, there was a significantly lower level of independence in skills related to “Preparing for Legal/Other Issues After Age 18”. Overall, level of independence/readiness seemed to not be influenced by patients’ age, gender, or disease type.

Discussion: Patients’ expectations and concerns about transition were mostly related to issues regarding patients’ perceived readiness for transition and the amount of support and understanding they receive from various health care providers. Additionally, continued monitoring and addressing of items in which the patient states he/she needs assistance with to achieve independence will be essential in the transition process.

Conclusions and Public Health Significance: Transition from pediatric to adult care providers is a crucial step in the care of individuals with sickle cell disease. By eliciting the expectations and concerns of these patients’ and evaluating their knowledge base and skills, we are able to gather further insight into barriers to transition, and begin to develop a plan to address these obstacles.

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PREFACE

It is incredible to think about all that I have accomplished during my graduate studies at the University Pittsburgh. In turn, I would like to thank my classmates and the staff/faculty of the Human Genetics Department for their friendship and guidance, without which I never would have made it through the past two years. I especially would like to express my overwhelming gratitude for Dr. Robin Grubs and the outstanding work she does as the Genetic Counseling Program Director.

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Lastly, I would like to thank my friends and family for their constant, unwavering support. My parents Vallita and Derrick and my sister Angela have always been there to provide me with unconditional love and encouragement to follow my dreams. As this journey comes to an end, I am excited to continue to make them proud as I prepare to begin what comes next!

1.0 INTRODUCTION

Sickle cell disease (SCD) is a genetic disorder that affects approximately 100,000 people in the United States (Molter & Abrahamson, 2014; Yawn, *et al.*, 2014). Although it can affect individuals of any ethnic/racial background, it is most often seen in individuals of African, Mediterranean, and Asian ancestry (Kanter & Kruse-Jarres, 2013). Individuals with sickle cell disease experience acute and chronic periods of pain, organ dysfunction, and a decline in quality of life and life span. Until the early 1970s, nearly 50% of children with sickle cell disease did not survive into adulthood (Andemariam, *et al*., 2014; Porter, *et al*., 2014). However, with advancements in comprehensive medical care, 93% now reach the age of 18 years (Lebensburger, *et al*., 2012), and the majority live into their 40's. This improvement in medical care for sickle cell patients, translating into improvement in survival, has created the need for an active and intentional process of transition from pediatric to adult oriented health care.

The Sickle Cell Clinic at Children's Hospital of Pittsburgh of UPMC (CHP) has been identifying children with sickle cell disease and providing families with counseling, follow-up care and further genetic testing since 1978. The clinic is staffed by an experienced team of medical professionals specialized in hematology/oncology, including a hematologist, physician assistant, nurse practitioners, nurses, social worker, genetic counselor, and behavioral health specialists. The goal of the Sickle Cell Clinic is to identify these children as early as possible, in order to effectively manage their condition into adulthood.

Furthermore, CHP's Sickle Cell Staff has recently taken steps to establish a transition program. This program will help patients and families to connect with adult care providers at UPMC's Adult Sickle Cell Program, which provides ongoing medical and psychosocial care for patients age 20 or older. The objective of this study was to conduct a preliminary assessment of the perspectives, current health care knowledge base and skills of patients with sickle cell disease in the pediatric setting as they prepare to transition towards adult health care, focusing on the knowledge and understanding that will be necessary to participate in the management of their sickle cell health care needs.

1.1 SPECIFIC AIMS

1.1.1 Specific Aim 1

To elicit adolescent and young adult sickle cell patients' perspectives regarding transition from pediatric to adult care in order to determine their expectations and concerns about the transition process.

1.1.2 Specific Aim 2

To evaluate the knowledge base and skill sets of adolescent and young adult sickle cell patients in regards to their self-management of sickle cell disease health care needs, including management of a chronic disease, insurance, doctor appointments, medications, and legal issues.

2.0 BACKGROUND AND SIGNIFICANCE

2.1 SICKLE CELL DISEASE

Sickle cell disease (SCD) refers to a group of inherited blood disorders (Hemoglobinopathies) characterized by the presence of abnormally shaped erythrocytes (red blood cells). Hemoglobin (Hb) is the protein found in blood that is responsible for transporting oxygen throughout the body. Additionally, hemoglobin plays a role in maintaining the shape of the erythrocytes. When examined under a microscope, erythrocytes normally appear round. However, individuals with sickle cell disease also have erythrocytes that appear crescent-shaped or sickled. The sickle-shaped erythrocytes have a shorter life span than normal erythrocytes. Additionally, they are more adherent and become lodged in blood vessels, thereby blocking the proper flow of blood. This results in the two key features of sickle cell disease, hemolysis and vaso-occlusion, which lead to tissue ischemia, pain crises, and multi-organ damage (Kanter & Kruse-Jarres, 2013).

2.1.1 Molecular Genetics and Pathophysiology

Hemoglobin consists of four globular protein subunits. In fetuses and infants, hemoglobin (Hb F) consists of 2 alpha-globin chains and 2 gamma-globin chains, $\alpha_2\gamma_2$. As the infant develops, the gamma-globin chains are slowly replaced by beta chains, forming the normal adult hemoglobin (Hb A), $\alpha_2\beta_2$. The α-globin chains are encoded by the the α-globin (*HBA*) gene found on

chromosome 16 and the β-globin chains are encoded by the β-globin (*HBB*) gene found on chromosome 11 (Clarke & Higgins, 2000).

Sickle hemoglobin (Hb S) results from a point mutation in the sixth amino acid of the *HBB* gene, which results in the formation of valine instead of glutamic acid (Glu6Val). Hb S results in erythrocytes with a lower oxygen affinity, and increased polymerization upon deoxygenation. This causes the erythrocytes to become misshapen (i.e. sickle-shaped), fragile (i.e. hemolysis), and adherent, leading to blockage in the blood vessels (i.e. vaso-occlusion).

The term sickle cell disease comprises a group of disorders characterized by the presence of at least one Hb S (β^S) allele and a second abnormal hemoglobin allele (Table 2). Homozygosity for β^S, HbSS, is referred to as sickle cell anemia (SCA) and is the most common and severe form of SCD. Sickle cell disease can also be inherited through compound heterozygosity of β^S and other mutant β-globins, most commonly hemoglobin C (Hb C). Hb C is formed from a point mutation in the sixth amino acid of the *HBB* gene, which causes the substitution of lysine from glutamic acid (Glu6Lys), and results in sickle cell hemoglobin C disease (HbSC). Other β-globin chain variants such as Hb D-Punjab (Glu121Gln), Hb O-Arab (Glu121Lys) and Hb E (Glu26Lys) are less common, and also result in sickle cell disease when co-inherited with Hb S (Bender & Douthitt Seibel, 2003; Clarke & Higgins, 2000). Mutations in the *HBB* gene that result in reduced amounts of (β^+) or absent (β^0) β–globin chains are referred to as Beta-Thalassemias, and when inherited with Hb S result in Sickle Cell Beta-Thalassemias (HbSβ^0 and HbSβ^+).

Table 1. Common Sickle Cell Disease Genotype-Phenotype Correlations

Name (Abbreviation)	Alleles Involved	Disease Severity	Characteristics
Normal (HbAA)	β and β	None	Hb A (Normal Adult Hemoglobin)
Sickle Cell Anemia (HbSS)	β^S and β^S	Severe	Hb S (Glu →Val) The most common and severe form of SCD
Sickle-Hb C Disease (HbSC)	β^S and β^C	Moderate	Hb S and Hb C (Glu →Lys)
Sickle-β^0-Thalassemia ($HbS\beta^0$)	β^S and β^0-Thal (severe mutation)	Severe	Hb S and Hb β^0-Thal (Absent β-globin chains)
Sickle-β^+-Thalassemia ($HbS\beta^+$)	β^S and β^+-Thal (moderate mutation) β^S and β^+-Thal (mild mutation)	Mild to Moderate	Hb S and Hb B^+-Thal (Reduced amounts of β-globin chains)

(Adapted from Bender & Douthitt Seibel, 2003; Clarke & Higgins, 2000)

2.1.2 Inheritance

Sickle cell disease is inherited in an autosomal recessive manner. For an individual to be affected, he/she must inherit mutations in both copies of the *HBB* gene (i.e. one from each parent). If an individual only has one mutation then he/she is said to have sickle cell trait (SCT), and are not affected. However, he/she is a carrier for SCD and has a 50% (or 1 in 2) chance of passing on the mutation to their child. When both parents are carriers than there is a 25% chance (or 1 in 4) that they will have an affected child, a 50% chance (or 1 in 2) that they will have a child who is a carrier of SCT, and a 25% chance (or 1 in 4) that the child will not be affected or a carrier.

2.1.3 Diagnosis

A diagnosis of sickle cell disease may be established through a variety of methods including hemoglobin electrophoresis, isoelectric focusing (IEF), high performance liquid chromatography (HPLC), and DNA analysis (Clarke & Higgins, 2000; Rees, 2010). Most often, an initial diagnosis is achieved through detecting significant amounts of Hb S through HPLC or IEF. Molecular genetic testing of the *HBB* gene through sequence analysis, deletion/duplication analysis, and targeted mutation analysis may be performed in order to confirm a diagnosis of sickle cell disease, test carriers, and perform prenatal diagnosis when there is a known familial mutation (Bender & Douthitt Seibel, 2003).

2.1.4 Clinical Manifestations and Management

As a result of the characteristic hemolysis and vaso-occlusion, sickle cell disease is a chronic, multisystemic condition associated with numerous health issues. Chronic hemolysis results in hemolytic anemia, which can manifest as pulmonary hypertension, priapism, and leg ulcers. Vaso-occlusion is associated with tissue ischemia that results in pain crises in the chest, abdomen, back, and extremities. These pain crises are frequent problems in pediatric sickle cell patients, are the most common reason sickle cell patients are admitted to the hospital, and are a significant source of morbidity and mortality (Rees, *et al.*, 2010; Wilson & Nelson, 2014). Treatment of pain crises in the clinical setting typically involves a combination of strategies including intravenous fluids, intravenous pain medication, and non-pharmacological techniques, such as heat packs and breathing exercises (Meier & Miller, 2012). Therefore, pain management

is multifaceted, and includes pharmacologic, physical, and psychological approaches (Wilson & Nelson, 2014).

Other complications include dactylitis (pain and/or swelling of the hands or feet), splenic sequestration and infarction, stroke, cerebral infarcts, intracranial hemorrhage, and kidney failure (de Montalembert, *et al*., 2014; Meier & Miller, 2012). Over time, these complications can lead to damage in numerous organs throughout the body including the spleen, lungs, kidneys, and bones. Damage to the spleen increases the risk of infection and sepsis, which in turn is the leading cause of death in children with sickle cell disease (Meier & Miller, 2012). Due to the increased risk of infections, standards of care include initiation of prophylactic penicillin upon diagnosis. Immunizations are also essential for those with sickle cell disease. Other complications of sickle cell disease often require treatment through proper hydration, pain medication, and blood transfusions (Wilson & Nelson, 2014).

Currently, Hydroxyurea is the first and only FDA approved medication for prevention of sickle cell disease related complications. The principle mechanism of this drug is that it increases the production of Hb F, which inhibits the polymerization of Hb S (Kanter & Kruse-Jarres, 2013). In turn, this reduces hemolysis and prevents acute vaso-occlusion. As a result, Hydroxyurea has been found to reduce the frequency of pain crises, acute chest syndrome, and transfusions by ~50% (Quinn, 2013). Previously used as a chemotherapeutic agent, side effects of Hydroxyurea are typically mild. These include dose-related leukopenia (decreased white blood cell counts) and thrombocytopenia (decreased platelet counts) (Rees, 2010). The side effects are reversible, and so patients who are on Hydroxyurea are closely monitored. Hematopoietic stem cell (or bone marrow) transplantation is the only curative treatment. Unfortunately, it is currently only recommended for children with HLA-compatible siblings. In

all, management of sickle cell disease requires a multidisciplinary team approach, in which care coordination is necessary to improve clinical outcomes and overall survival of patients (Hankins, *et al*., 2012).

2.2 TRANSITION

2.2.1 Background

In an American Academy of Pediatrics (AAP) Survey performed in 2008, pediatric providers noted that 84% of youth/young adults with special health care needs lacked knowledge about their medical condition, and only 12% of the providers surveyed created individualized transition plans for their patients (McManus, *et al*., 2008). In one of the first studies of sickle cell disease health care transition, issues, concerns, and expectations of adolescents and young adults, parents or guardians were investigated as they related to the transfer to adult care. The young adults with sickle cell disease were concerned about how they would pay for medical care and how they would be treated in the adult setting (Bryant & Walsh, 2009). Their caregivers were concerned regarding the youth's ability to take on responsibility for self-care and cope successfully in the adult health care system. In a multi-site study of sickle cell transition in sickle cell centers and community-based SCD programs, the majority of patients voiced fear of leaving their pediatric health care providers, concerned that the adult providers might not understand their needs and might not believe their complaints of pain (Cerns, *et al.*, 2013).

Often, in the past, age has determined when transfer of care has occurred, rather than an individual assessment of patient readiness or compliance (Sobota, *et al*., 2011). Data reveals

little progress has been made in transition from pediatric to adult health care in the last decade despite a 2002 Consensus statement by the American Academy of Pediatrics (AAP)/American Academy of Family Physicians (AAFP)/American College of Physicians (ACP) which included the identification of six critical steps to ensuring successful transitioning to adult-oriented health care. These steps consisted of: "1) ensuring that all young people with special health care needs have an identified health care professional who attends to the unique challenges of transition, 2) identifying the core knowledge and skills required for transition, 3) preparing and maintaining an up-to-date medical summary that is portable and accessible, 4) creating a written health care transition plan by age 14 together with the young person and family, 5) applying the same guidelines for primary and preventive care for all adolescents and young adults, including those with special health care needs, and 6) ensuring affordable, continuous health insurance coverage" (American Academy of Pediatrics, *et al.*, 2002).

2.2.2 Significance

In 2011, a clinical report by the AAP, AAFP, and the ACP was published which provides practical, detailed guidance with an algorithm on how to plan and implement better health care transitions for all patients. Furthermore, it integrates transition planning into the medical home and into ongoing chronic care management through identification of six core elements of health care transition: 1) transition policy, 2) transition tracking and monitoring, 3) transition readiness, 4) transition planning, 5) transfer of care, and 6) transfer completion (American Academy of Pediatrics, *et al.*, 2011). When asked, 62% of pediatric providers thought transition planning for their patients should begin at 18-20 years of age for those patients with special needs, and 65% of pediatric providers said this same age bracket would be the best time to begin transition

planning for those patients without special needs (McManus, *et al*., 2008; Cooley & Sagerman, 2011). Yet, the literature shows transition planning should begin at a much earlier age between 13-14 years old for the best outcome (American Academy of Pediatrics, *et al*., 2011; Treadwell, *et al.*, 2011). All youth must navigate rapidly changing, physical, psychological and social issues as they move forward towards independence, but those with sickle cell disease, as well as other chronic medical conditions, will need additional support as they must also adjust to taking on increasing responsibility in the management of what is a lifelong condition (Cerns, *et al.*, 2013; van Staa, *et al.* 2011; Wills, *et al.*, 2010). So, it is believed that preparation for transition should start early and focus on strengthening adolescent independence without undermining parental involvement (van Staa, *et al.* 2011).

2.2.3 Establishment of a Transition Program

According to the Centers for Disease Control and Prevention's (CDC) national resource directory, as of April 2013 there are 184 pediatric and 54 adult providers/sickle cell centers nationwide. This data would indicate that most adult sickle cell patients are cared for outside of a designated sickle cell center. Additionally, in 2011, a multi-site study of pediatric sickle cell clinics was conducted. The aim of this study was to describe the current transition practices of these clinics in order to identify areas for improvement. Of the 30 pediatric sickle cell clinics surveyed, only 18 reported that they routinely transferred their patients to an adult hematologist specializing in sickle cell disease (Sobota, et al., 2011).

In an effort to provide a smooth and successful transition process from pediatric to adult oriented care, CHP's Sickle Cell Staff has recently taken steps to establish a transition program. The planned transition program will focus on health literacy, disease self-management skills,

improving communication skills with health care providers, and life planning. It will implement a newly designed survey tool, in conjunction with a motivational interviewing style, to assess and evaluate the program. The survey tool is a four part questionnaire that contains a portable medical summary in addition to a readiness assessment tool, which will be updated at each visit. The portable medical summary prevents duplication of test and procedures, and helps streamline the information gathering process for providers. This in turn improves patient safety, and ensures adult providers have accurate information. Motivational interviewing will be conducted by a trained health care professional who is a member of the research team, and will be utilized during transition clinic visits. This is a patient-centered approach that will be used with the intent to elicit and strengthen the patient's own motivations for behavioral changes towards successful transition. Motivational interviews will be audio taped for transcription and analysis. It is expected that some conversations may reveal more in-depth understanding of patients' needs than what the survey tool is able to capture.

By implementing this transition program, the Sickle Cell Clinic hopes to achieve greater involvement from patients and families so they may learn how to utilize their own skills and abilities in managing their health care and health care needs, as well as strengthening the independence of youth/young adults with sickle cell disease as they move forward towards maturity with regards to their own health care. This survey instrument also allows for the collection of data regarding the perceptions of sickle cell patients being seen at Children's Hospital of Pittsburgh, who are 15 years of age and older, about the process of transition from pediatric to adult medical care for their sickle cell disease.

3.0 STUDY DESIGN AND METHODS

3.1 PROJECT DEVELOPMENT

This project was conducted through collaboration with the Sickle Cell Clinic at Children's Hospital of Pittsburgh of UPMC (CHP). As part of the establishment of the transition program, an ongoing study will be conducted that will collect data regarding the transition process of medical care for sickle cell patients from pediatric to adult oriented care. The study was most recently reviewed and approved by the University of Pittsburgh Institutional Review Board on August 13th 2014 identified as IRB #PRO13030461 (See Appendix A). Prior to the full establishment of the clinic's transition program, an initial round of transition questionnaires were distributed to eligible patients who had agreed to be in the study. This project was designed as a substudy to conduct a preliminary assessment of these previously collected questionnaires to determine patients' perspectives on transition and the level of independence they had regarding various skills essential to transition and self-management of their sickle cell health care needs.

3.2 PATIENT POPULATION

The patient population consisted of male and female patients who were evaluated by members of the research/health care team of the Sickle Cell Clinic at Children's Hospital of Pittsburgh.

Inclusion criteria consisted of being a patient with sickle cell disease between the ages of 15-22 years seeking care at the CHP Sickle Cell Clinic. An exclusion criterion was any patients under the age of 15.

3.3 PATIENT RECRUITMENT

Patients of the Sickle Cell Clinic at the Children's Hospital of Pittsburgh must transfer to adult care by the age of 22 years. As transition is an active process that requires gaining independence, which can only take place over time, it is suggested that the patients begin the process of transition at 15 years of age. Patients age 18 to 22 years and parents of patients aged 15 to under 18 years who receive sickle cell health care at the CHP Sickle Cell Clinic were mailed letters via the US postal service introducing them to the study. This letter was sent by members of the clinic's research team, which includes the physician, social worker, case manager, and nurse practitioner, all of whom are known to the patients who seek care at the CHP Sickle Cell Clinic. Patients were then approached by a member of the research team, who is also part of the health care team, during a regularly scheduled clinic appointment. Patients and parents as applicable received an explanation of the study, and were offered the opportunity to enroll in the study after review of consent.

3.4 INFORMED CONSENT

A member of the research team provided an introduction/review of the research study, including potential risks and benefits, protocol procedures, and research team expectations. Patients, and as applicable parents/legal guardians were encouraged to ask questions. Patients/parents were given the opportunity to speak to the principal investigator (PI) prior to enrollment, if requested. Consent was then obtained from patients 18 years of age and older, and from a parent/legal guardian for patients under 18 years of age following the patient's assent. A copy of the signed consent was given to all patients, as well as parent/legal guardian as applicable.

3.5 SAMPLE

At the time of the study, the CHP Sickle Cell Clinic had 30 patients identified as meeting the inclusion criteria, and 25 of these individuals consented to being in the study. Of these 25 patients, 18 of them completed the portions of the self-administered questionnaire pertaining to their thoughts on transition and skill sets.

3.6 SURVEY TOOL

Patients with sickle cell disease being treated at the Sickle Cell Clinic at Children's Hospital of Pittsburgh who were between the ages of 15-22 years, and who had consented to being in the study received a self-administered four part questionnaire during a regularly scheduled visit. The

questionnaire was created by members of the CHP Sickle Cell Program, and was adapted for the sickle cell population from the customizable, standardized readiness assessment tool provided by Got Transition/Center for Health Care Transition (A cooperative agreement between the Maternal and Child Health Bureau and the National Alliance to Advance Adolescent Health). The customizable readiness tool has been assessed by the National Quality Forum's measure evaluation criteria and process, and was found to have good internal reliability and criterion validity (McPheeters, *et al*., 2014; National Health Care Transition Center, 2015).

The four part questionnaire consisted of a portable medical summary and patient readiness tool. The portable medical summary (section 1) included questions regarding demographic information. The patient readiness tool was subdivided into two fill in the blank sections regarding the patients' thoughts on transition and their strengths (sections 2 and 3), as well as a quantitative section regarding their skill sets (section 4). This project focused solely on the portions of the transition questionnaire regarding patients' thoughts on transition (section 2) and their various skill sets (section 4).

Section 2: Thoughts About Transition (See Appendix B)

Consisted of five open-ended questions inquiring about patients' thoughts on transition.

Section 4: Skill Sets (See Appendix C)

Consisted of thirty-eight close-ended questions that elicited information pertaining to four categories: 1) Knowledge of Health Issues/Diagnosis, 2) Being Prepared, 3) Taking Charge, and 4) Preparing for Legal/Other Issues After Age 18. Answers were recorded on a 3-point Likert scale ("I always depend on other to do this for me", "I am currently learning to how to do this independently", "I can do this independently") and were scored from 1 to 3.

3.7 DATA ANALYSIS

Section 2: Thoughts About Transition

The open-ended questions inquiring about patients' perceptions on transition resulted in qualitative data, which was evaluated using thematic analysis. Responses were reviewed and analyzed to determine repeating themes. This was done by coding and interpreting the data through a series of steps involving: 1) reading through all the responses, 2) developing categories, 3) assigning each response to a category, 4) checking the categories, 5) reviewing for major themes, 6) identifying patterns and trends, and 7) writing-up the analysis (Braun & Clark, 2006).

Section 4: Skill Sets

Likert scales were used in order to quantify the level of independence of the various skills pertaining to transition from pediatric to adult care. The overall level of independence of each patient was analyzed by taking each response from the 38 skill set questions, converting them to a numerical value (1-3), and then finding the average of these values. The average level of independence of the patients were then compared across the various skills set, and demographic characteristics (i.e. age, gender, and disease type/severity). Non-parametric tests were used for these comparisons given the small sample size and the assumption that the data would not follow a normal distribution. The Mann-Whitney test was used for comparisons of the medians between two groups (i.e. by age and gender), while the Kruskal-Wallis test was used for comparisons of the medians between more than two groups (i.e. between the skill sets and by disease type). All statistical analysis was carried out using Microsoft ® Excel and Minitab 16.0 statistical software.

4.0 RESULTS

4.1 DEMOGRAPHICS

A total of 18 adolescents and young adults with sickle cell disease were surveyed (Table 2).

Table 2. Summary of Patient Characteristics

Variables	**N (%)**
Sex	
Male	3 (16.7)
Female	15 (83.3)
Race/Ethnicity	
African American	18 (100)
Age (y)	
<18	5 (27.8)
18-22	13 (72.2)
Average age (y)	18.6
Sickle Cell Diagnosis	
HbSS	6 (33.3)
HbSC	8 (44.4)
HbSβ^0Thal	0 (0)
HbSβ^+Thal	3 (16.7)
Unknown	1 (5.6)

The majority of patients were 18 years or older (72.2%), with an average age of 18.6 (SD = 2.1) years. Among the patients, 15 (83.3%) were female and 3 (16.7%) were male. All patients were of African American racial/ethnic background. As far as sickle cell disease type, 6 (33.3%) had HbSS, 8 (44.4%) had HbSC, 3 had (16.7%) HbSβ^{+}Thal, and 1 (5.6%) individual did not report on their sickle cell disease type, and so their diagnosis is unknown. (See Figures 1-5).

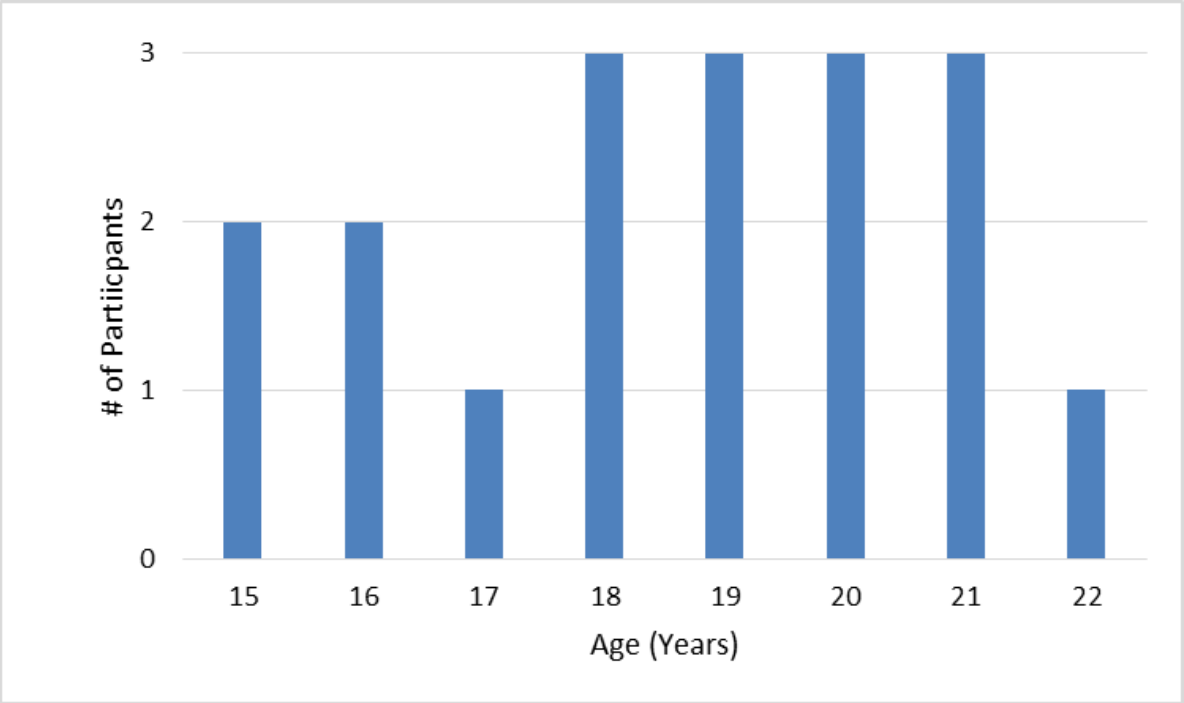

Figure 1. Age Distribution of Patients

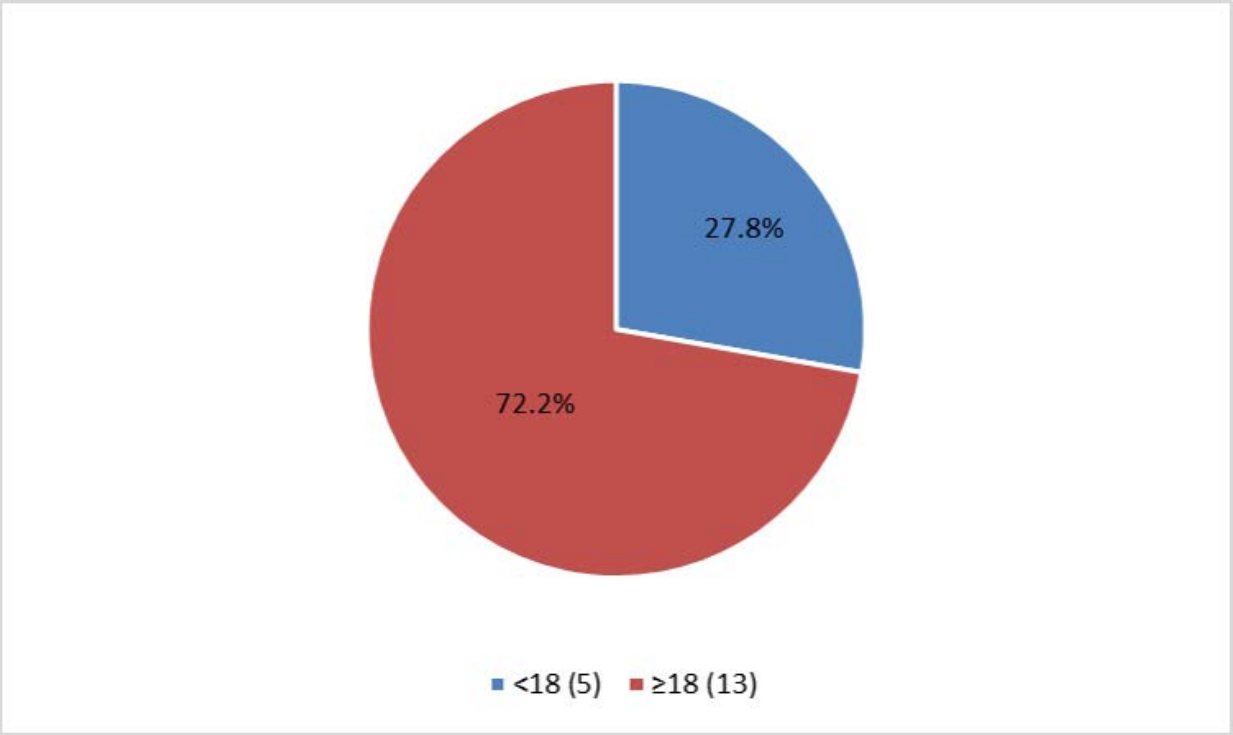

Figure 2. Age Group Distribution of Patients

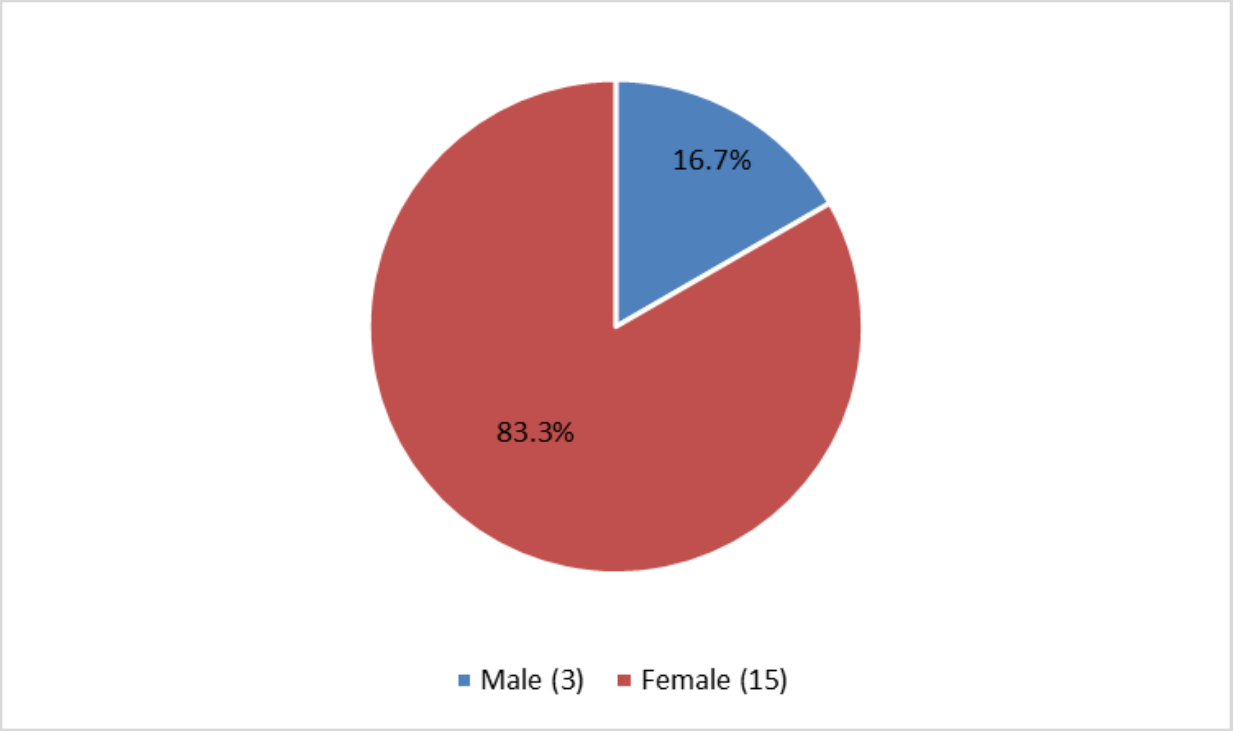

Figure 3. Gender Distribution of Patients

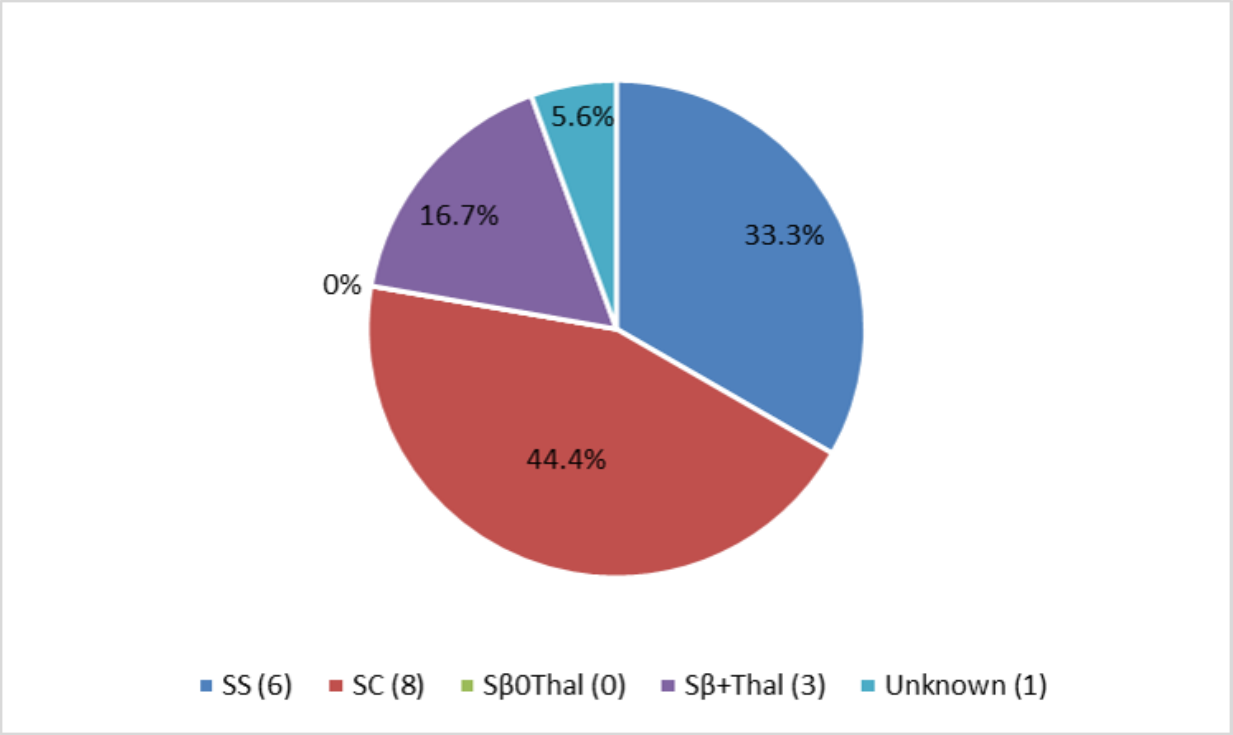

Figure 4. Disease Type of Patients

4.2 THOUGHTS ABOUT TRANSITION

Of the 18 patients surveyed, 17 (94.4%) answered the questions in section 2 of the transition questionairre. Table 3 displays the responses to the five open-ended questions regarding transition.

Table 3. Patients' Responses to Open-Ended Questions Regarding Transition

What are the good things about transitioning?	What are the not so good things about transitioning?	What is the best thing that will happen to you when you will transition?	What is the worst thing that you see happening you to when you transition?	Can you state at least one thing that you have done in the past 6 months to improve the transition process?
You are treated as you should be at your age	Less comfortable with the doctors and the hospital	Not sure	The new treatment team will be compassionate	Attend a clinic
I'll be in an adult hospital	None of the people I'm used to	I'll be more of an adult	People don't adjust too well	Made it to my appointments
I will be better equipped to handle myself in the future	I can' t think of anything	I'll be able to go anywhere	Nothing	No
IDK	IDK	IDK	IDK	IDK
No idea	New doctors and nurses	I have no clue	Nothing	No
Knowing that I am growing up and healthy	Having to leave the hematology team that I have grown to know and love	Getting a new team of doctors	Having to explain things that my pediatric team already knows, or not being understood	Make my appointment!
I don’t know - nothing	Losing my doctors [that] helped me grow up	I somehow will get to keep my doctor	I lose my doctor	Came to clinic
Getting to know new people	None	I don’t know	None	None
No response	No response	No response	No response	No response
It helps patients become more independent and knowledgeable	Nothing I can think of	I will be more independent	I will try to solve a crisis on my own	I write down my medications and doses, my doctors and their numbers, and my appointments

Table 3 Continued

Valuable information being shared about sickle cell, known and unknown facts	Leaving the wonderful care team I have grown to love	Moving forward with my illness	N/A	Stay in contact with doctors
Good experience	Different surroundings	No response	Being on my own at the hospital	No response
It's good because your moving onto The adult world, getting older and growing wiser	You're leaving children's hospital Where they know me best and [have] work with most of your life and have to meet a new team of doctors	The same day clinic	Transitioning too early, not being Ready to move forward to the adult world	Transition classes
Welcomes me to the real world/adulthood	We knew all the doctors and nurse well	I'm not sure	Not getting along with nurses and doctors	No
I can experience adulthood	Meeting new people	IDK	The doctors not knowing me very well	No
Nothing	Don't know the people/staff, no Disney movies, pay for tv, not as fun food	Can't think of anything	Mistreatment	I showed up today
Be more independent, handle my medication on my own	Scary, probably less support than with CHP staff	Can't think of anything	That I will be alone, I will not be able to talk to you guys	Come to clinic
The good things are the excitement of having a new doctor and new experiences	The doctors not knowing me personally and not understanding my life outside of the hospital	I will have a new experience with someone new	Awkward moments between me and my new doctor	Have not started yet

After reviewing the responses, most of the answers the patients gave about their expectations and concerns regarding transition appeared to fall into two categories: health care-related issues and patient-related issues. Amongst these two categories, four themes were identified: 1) Apprehension to Leaving Pediatric Care, 2) Forming New Relationships with Adult Providers, 3) Growing Up and Responsibility, and 4) Worries Regarding Readiness and Support.

4.2.1 Health Care-Related Issues

Apprehension to Leaving Pediatric Care

When asked to name some of the not so good things regarding transition, seven of the patients mentioned leaving their current health care team. Responses included:

"Having to leave the hematology team that I have grown to know and love",

"Losing my doctors [that] helped me grow up", and

"You're leaving children's hospital where they know me best and [have] work with most of your life and have to meet a new team of doctors".

Two patients stated that no longer having their current doctor/health care team was the worst thing about transition, claiming:

"*Having to explain things that my pediatric team already know, or not being understood"*, and

"I lose my doctor".

Forming New Relationships with Adult Providers

Another theme that appeared to reoccur was patients' perspectives on forming new relationships with adult providers. Patients made comments that were both positive and negative regarding this theme. When asked what some of the good things about transitioning were, two patients replied,

"Getting to know new people", and

"The good things are the excitement of having a new doctor and new experiences".

Additionally, two patients mentioned that the best thing that will happen when they transition is:

"Getting a new team of doctors", and

"I will have a new experience with someone new".

On the other hand, this was mentioned as one of the not so good things about transition by five patients. Comments from these individuals included:

"Less comfortable with the doctors and the hospital", and

"The doctors not knowing me personally and not understanding my life outside of the hospital".

Furthermore, not having an established relationship with adult health care providers was reported as the worst part of transitioning by three of the patients surveyed. These individuals expressed concern about:

"Not getting along with nurses and doctors",

"The doctors not knowing me well", and

"Awkward moments between me and my new doctor".

4.2.2 Patient-Related Issues

Growing Up and Responsibility

A theme that appeared to be a positive aspect of transitioning was the move into adulthood and gaining independence. Nine patients reported this issue as one of the good things about transition, stating that:

"You are treated as you should be at your age",

"It helps patients become more independent and knowledgeable", and

"It's good because you are moving onto the adult world, getting older and growing wiser".

Additionally, it was mentioned as the best part of transition by four patients. Responses included:

"I'll be more of an adult", and

"Moving forward with my illness".

Worries Regarding Readiness and Support

A theme that appeared to be an overwhelming negative aspect of transitioning was worries regarding readiness for transition and the level of support that will be in place. This theme was mentioned as one of the not so good things about transition by a patient, who stated:

"Scary, probably less support than with CHP staff".

Additionally, it was mentioned as the worst part of transition by five patients. Responses included concerns that:

"People don't adjust too well",

"That I will be alone",

"I will try to solve a crisis on my own", and

Transitioning too early, not being ready to move forward to the adult world".

4.2.3 Active Role in Transition

Lastly, patients were asked if they could state one thing that they have done in the past 6 months to improve the transition process. Of the ten patients who answered this question in the affirmative, all of them mentioned something that involved them taking an active role in their health care management. This was done either by attending their appointments/clinic, being in contact with their doctors, or writing down their important health information (i.e. medications and doses, appointments, etc.).

4.3 KNOWLEDGE BASE AND SKILL SETS

4.3.1 Knowledge of Health Issues/Diagnosis

Figure 5 summarizes the results from the 14 “Knowledge of Health Issues/Diagnosis” questions. The majority of patients, 88.9%, could explain the type of sickle cell disease they have and 72.2% stated that they understood their diagnosis and health history, and could explain it to others. 83.3% reported that they know and can state the significant health problems caused by their sickle cell disease, while only 61.1% knew what their other non-sickle cell diagnoses were. In regards to communicating with a new provider, 77.8% reported that they could explain their health care needs and medical treatments, while 83.3% could state what medications they have been prescribed. Additionally, 88.9% of patients knew how to pick up their medicine from the pharmacy. 55.6% knew when they last saw each specialist, and only 27.8% knew their hemoglobin and reticulocyte level baseline, as well as their pulse oximeter baseline number. However, 94.4% recognized which of their symptoms needed quick medical attention, and what to do in case they have a medical emergency. It is important to note that this skill set measured patients’ perception of their knowledge, and not their actual level of knowledge.

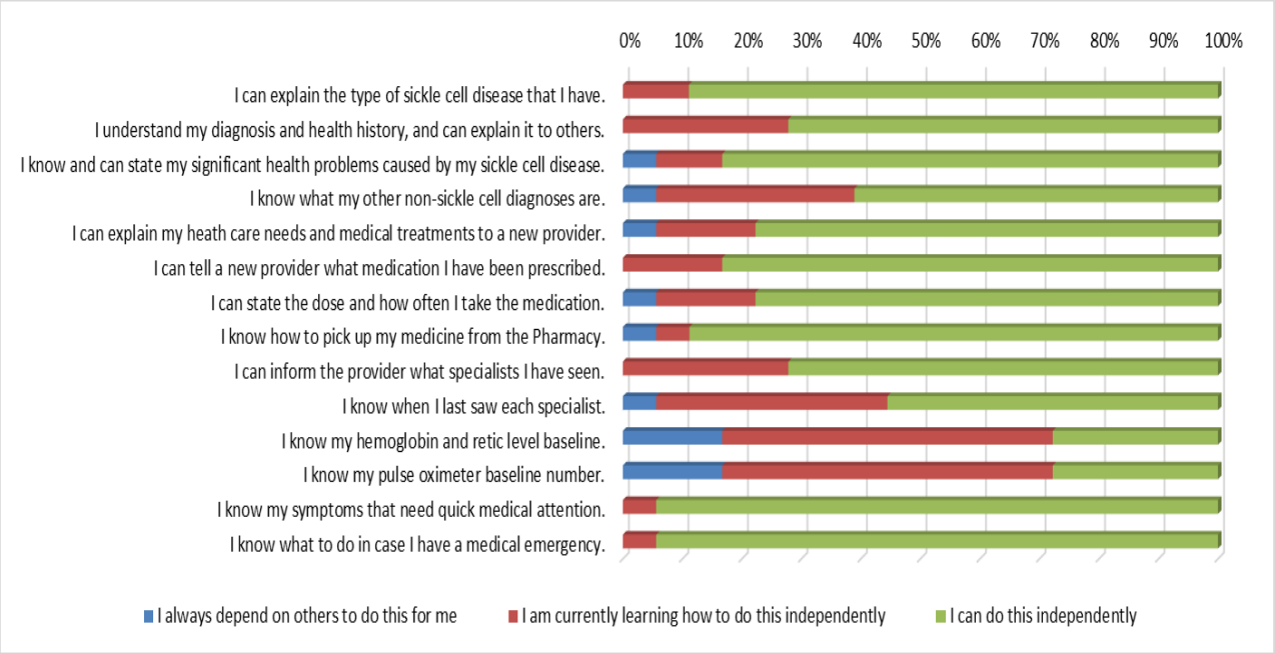

Figure 5. Patients' Self-Reported Levels of Independence Regarding Knowledge of Health Issues/Diagnosis

4.3.2 Being Prepared

Figure 6 summarizes the results from the 4 “Being Prepared” questions. 72.2% of patients reported that they carry their health insurance card every day, and 66.7% knew what type of medical coverage they have. When asked if they either carry or know where to get their important health information, 55.6% of patients stated that they did. Lastly, of the patients that were 18 years or older, 46.2% claimed that they have an adult primary care physician.

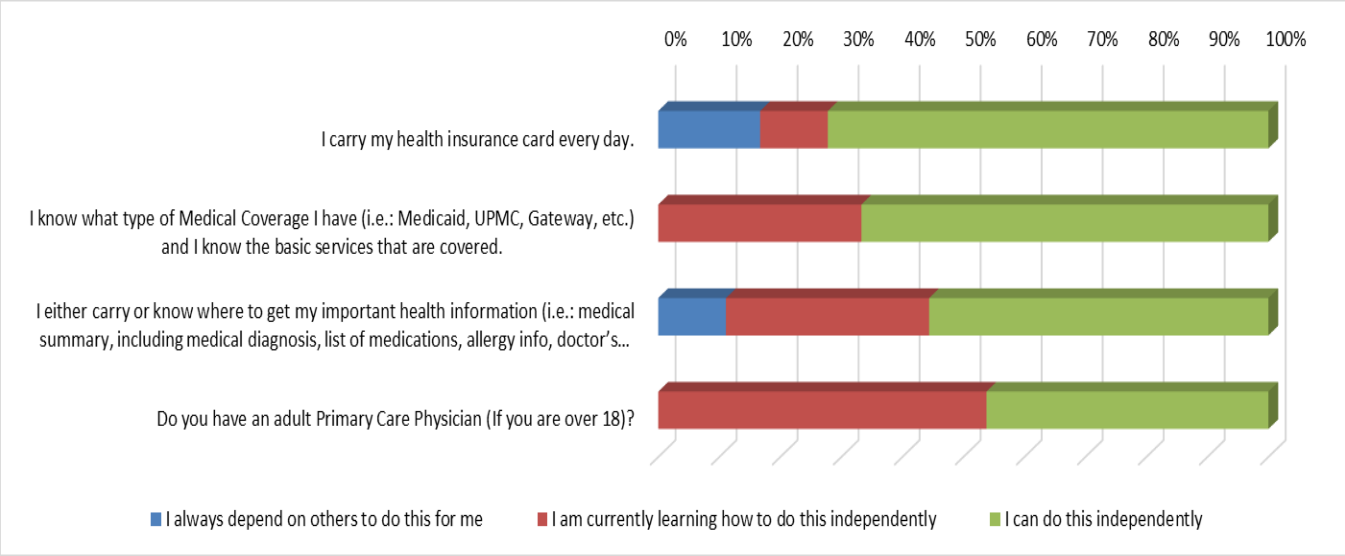

Figure 6. Patients' Self-Reported Levels of Independence Regarding Being Prepared

4.3.3 Taking Charge

Figure 7 summarizes the results from the 13 "Taking Charge" questions. 72.2% of patients stated that they call and make their own doctors' appointments, and 88.9% use a cell phone or calendar to remind them of their appointments and medication refills. 77.8% reported that they knew that they have an option to see their doctor by themselves, and 61.1% prepare written questions to ask prior to a doctor's appointment. Additionally, 94.4% said that they could read a prescription label. However, only 50% track their own appointment and prescription refill expiration dates, and 66.7% call in their own prescriptions refills. 88.9% can state where they typically experience pain during a pain episode, and 72.2% know how to manage their pain and diagnosis at home. 94.4% know what medications help to manage their pain, and how to take these medications. Furthermore, 72.2% know what non-medication steps to take to help manage their pain, and 61.1% know stress reduction or relaxation techniques that help main their pain.

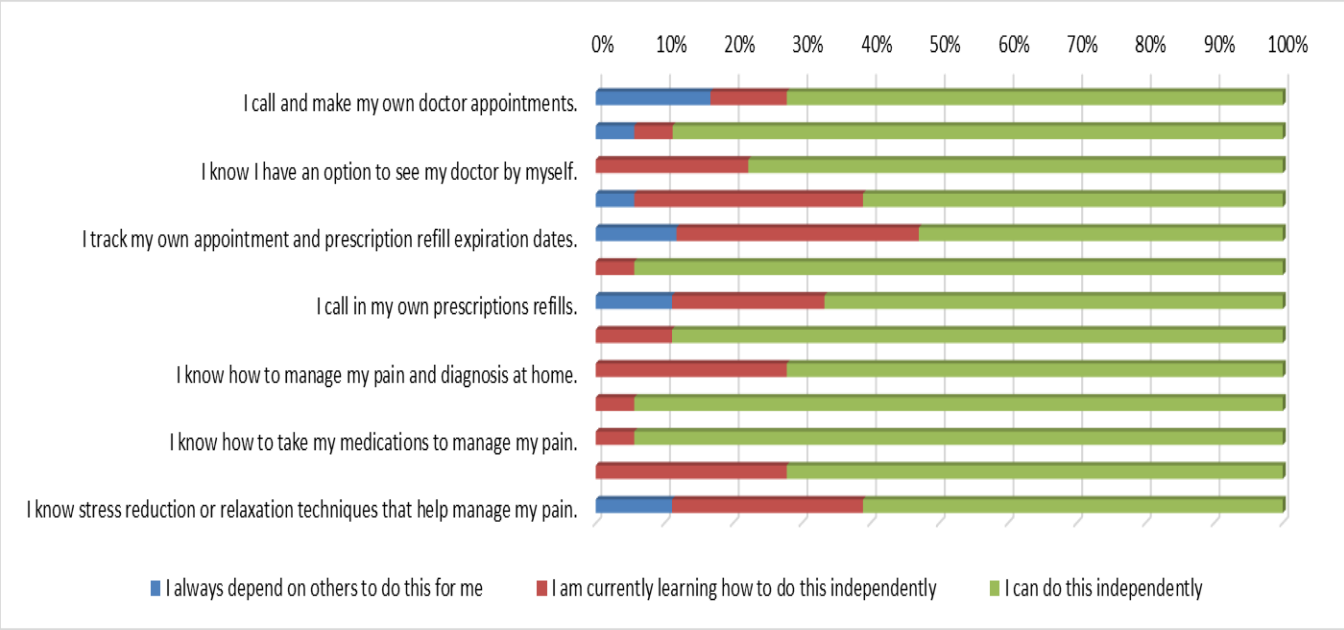

Figure 7. Patients' Self-Reported Levels of Independence Regarding Taking Charge

4.3.4 Preparing for Legal/Other Issues After Age 18

Figure 8 summarizes the results from the 7 "Preparing for Legal/Other Issues After Age 18" questions. 44.4% of patients stated that they have a plan to keep their health care insurance or obtain medical coverage after they turn 18. 27.8% reported that they have a plan to develop a legal Power of Attorney for health care decisions in the event that their health changes and they are unable to make decisions for themselves. 38.9% claimed that they knew of resources that can help them to find adult services (i.e. job support, transportation, assistive technology, etc). 44.4% stated that they knew how their condition may affect job choices, and 38.9% knew what benefits they might qualify for (i.e. SSI, community based services, etc). Lastly, 55.6% said they knew their options for housing as an adult, and 44.4% knew how to manage their money and pay their bills.

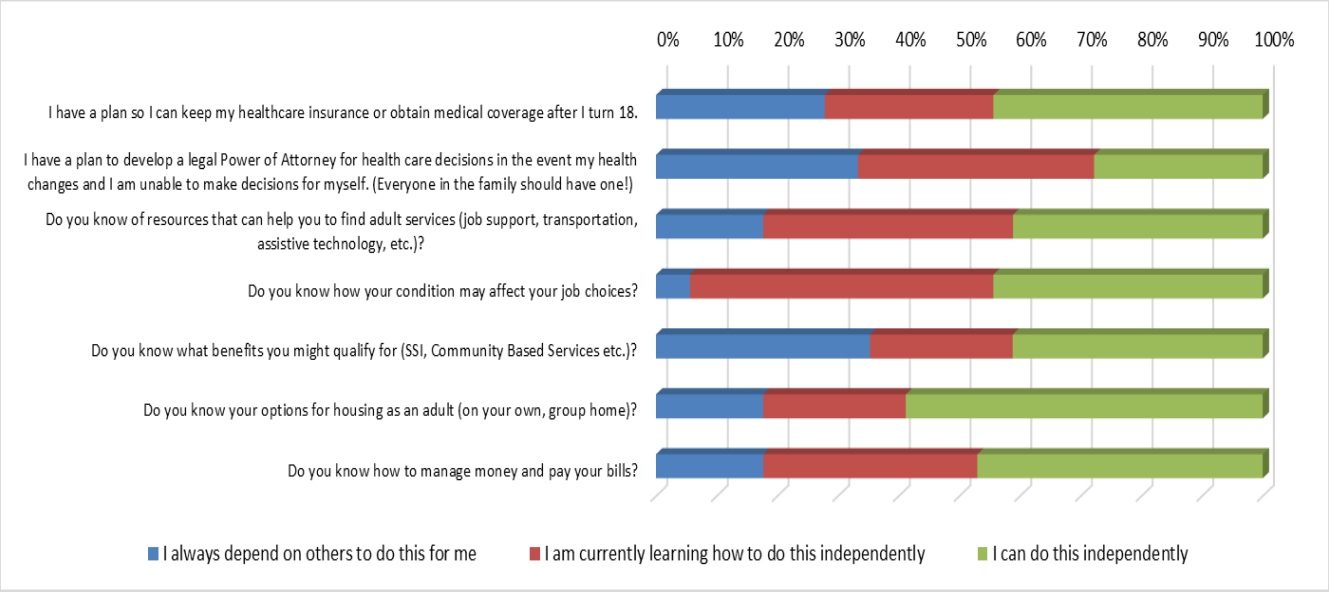

Figure 8. Patients' Self-Reported Levels of Independence/Readiness Regarding Preparing for Legal/Other Issues After Age 18

4.4 INDEPENDENCE/READINESS ASSESSMENT

4.4.1 Responses by Skill Set

The medians of the average levels of independence for each of the skill sets were compared to each other, and were 2.72, 2.51, 2.72, and 2.24 for Knowledge of Health Issues/Diagnosis, Being Prepared, Taking Charge, and Preparing for Legal/Other Issues After Age 18, respectively (Figure 9). The Kruskal-Wallis test was used to compare these groups. The distribution amongst the four groups did differ significantly ($H = 16.06$, p-value (0.001) < 0.05). The level of independence in doing the tasks mentioned in the "Preparing for Legal/Other Issues After Age 18" skill set was significantly lower than those in the other skill sets.

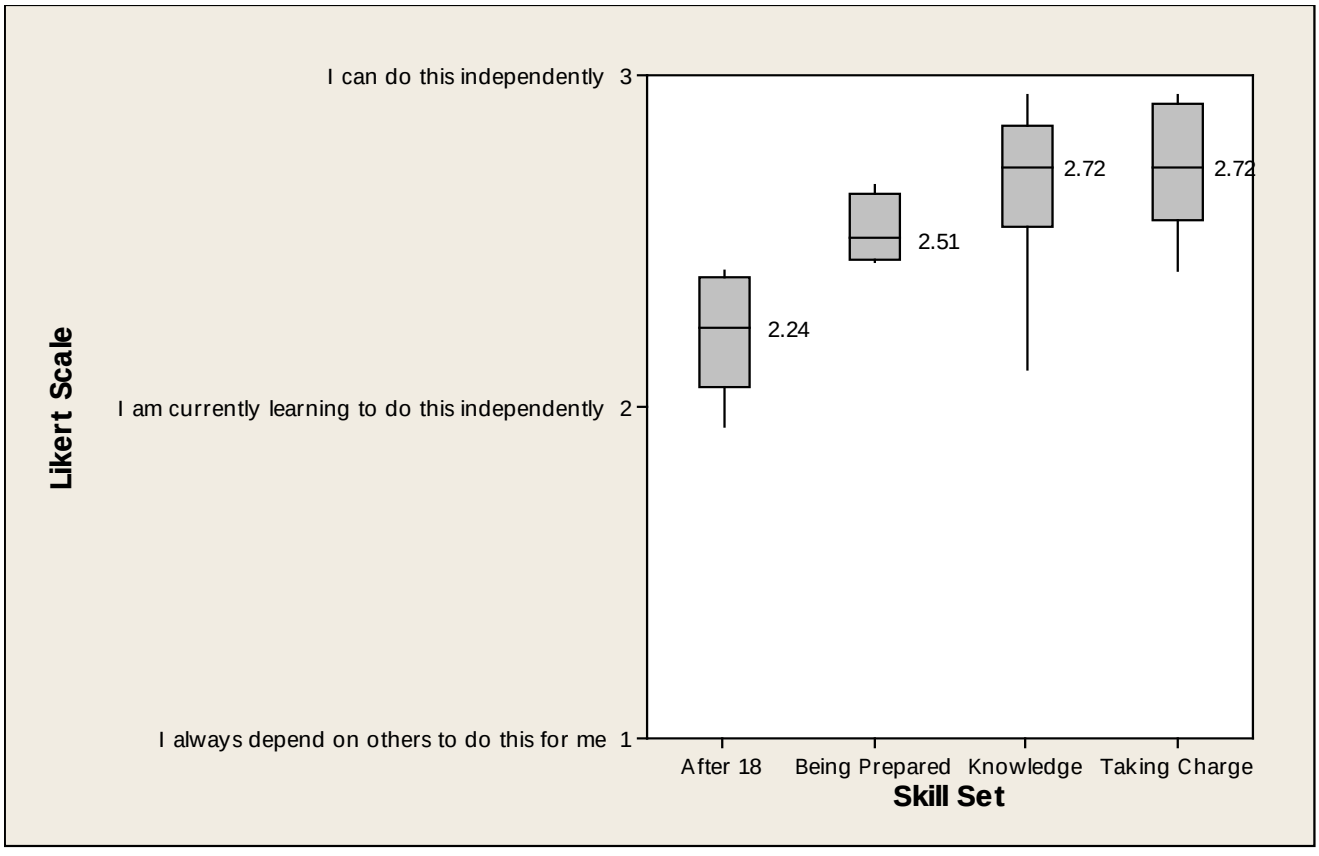

Figure 9. Comparison of Patients' Independence/Readiness Across the Various Skill Sets

4.4.2 Responses by Age

The median levels of independence were 2.43 and 2.68 for under 18 years of age and ≥18 years of age and older, respectively (Figure 10). The Mann-Whitney test was used to compare these two groups. The distribution of these two groups did not differ significantly (W=30.5, p-value (0.1039) > 0.05).

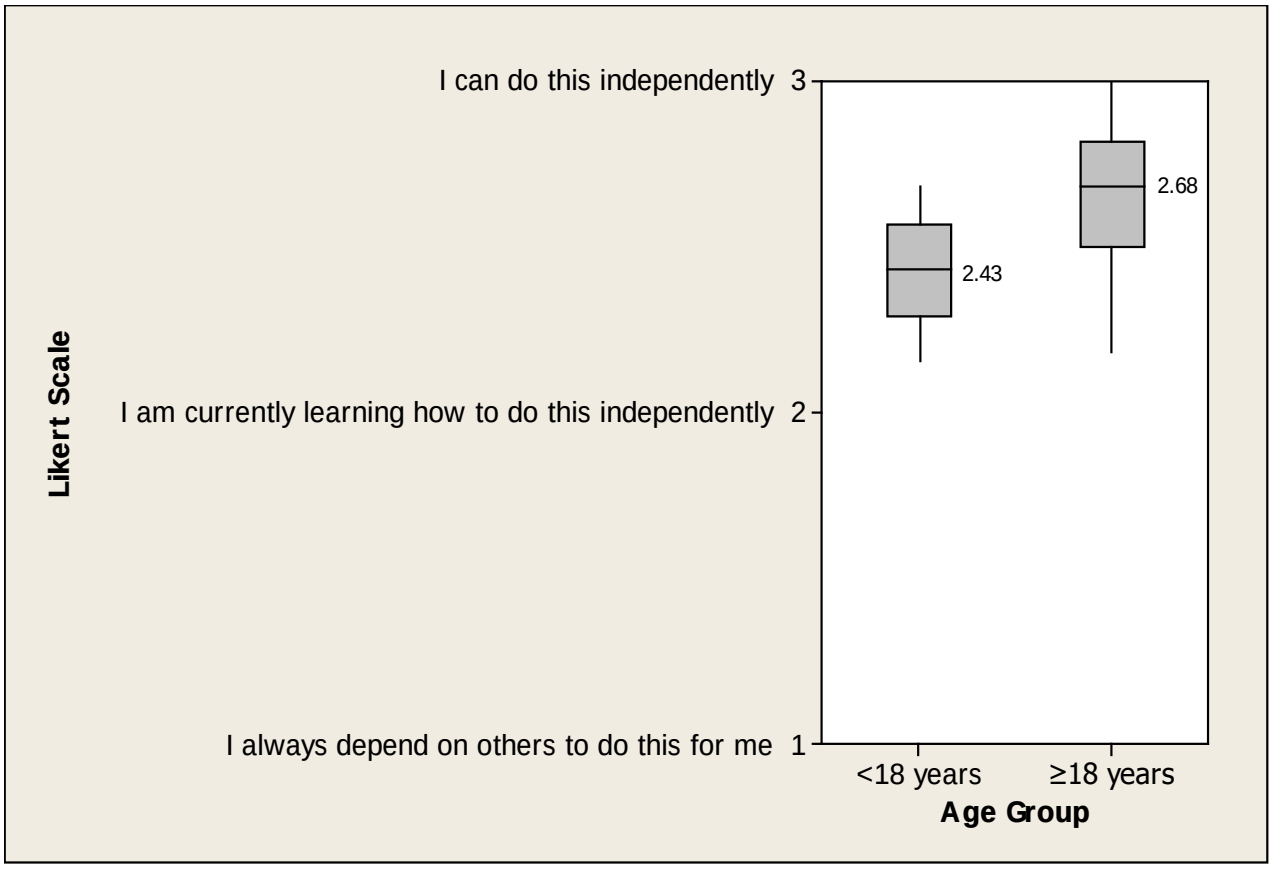

Figure 10. Comparison of Patients' Independence/Readiness by Age

4.4.3 Responses by Gender

The median levels of independence for females and males were 2.63 and 2.61, respectively (Figure 11). The Mann-Whitney test was used to compare these two groups. The distribution in the two groups did not differ significantly (W=28.5, p-value (1.0000) > 0.05).

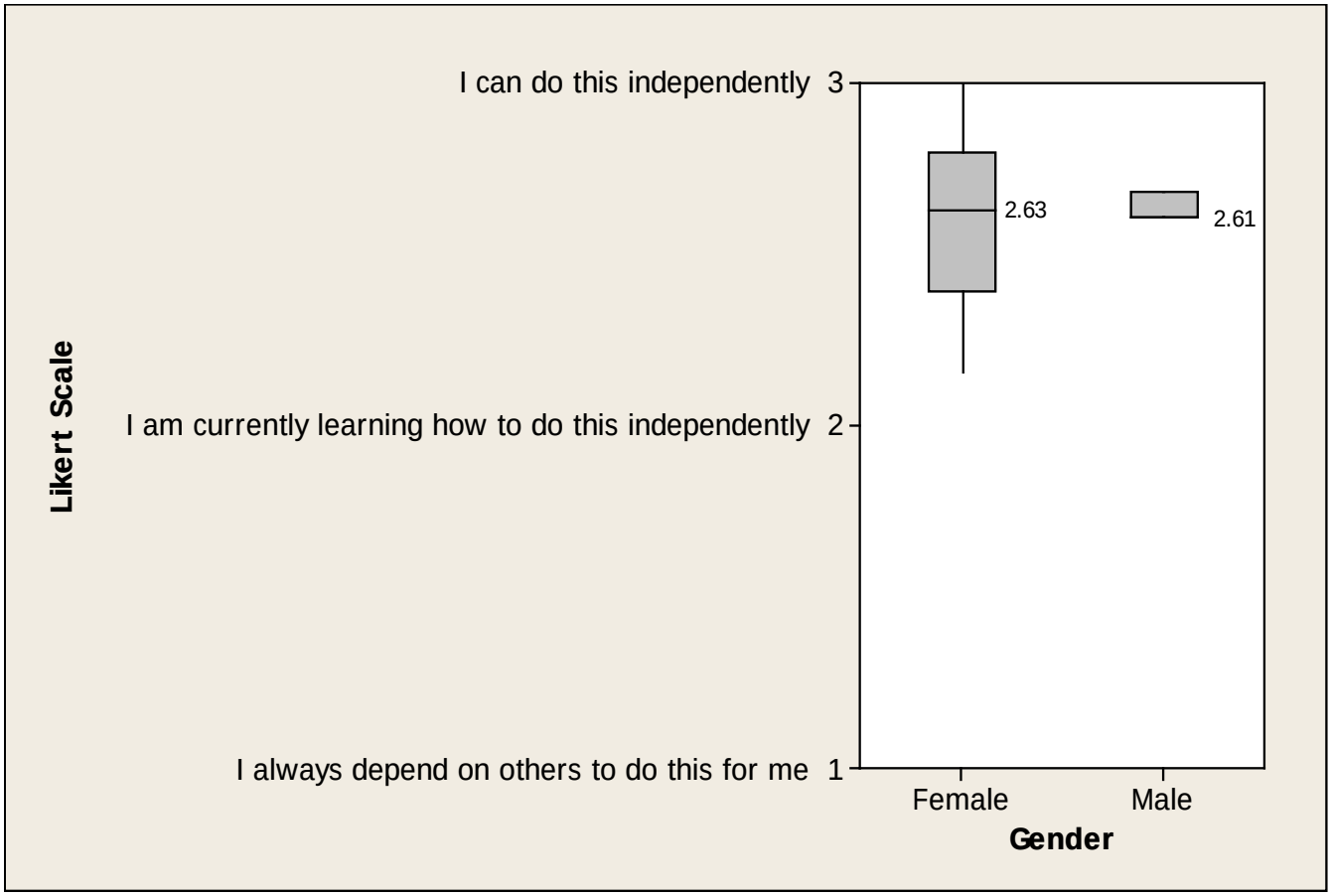

Figure 11. Comparison of Patients' Independence/Readiness by Gender

4.4.4 Responses by Disease Type/Severity

The median levels of independence were 2.66, 2.43, 2.84, and 2.43 for SS, SC, Sβ^{+}-Thal and unknown, respectively (Figure 12). The Kruskal-Wallis test was used to compare these groups. The distribution amongst the four groups did not differ significantly (H = 2.42, p-value (0.490) > 0.05).

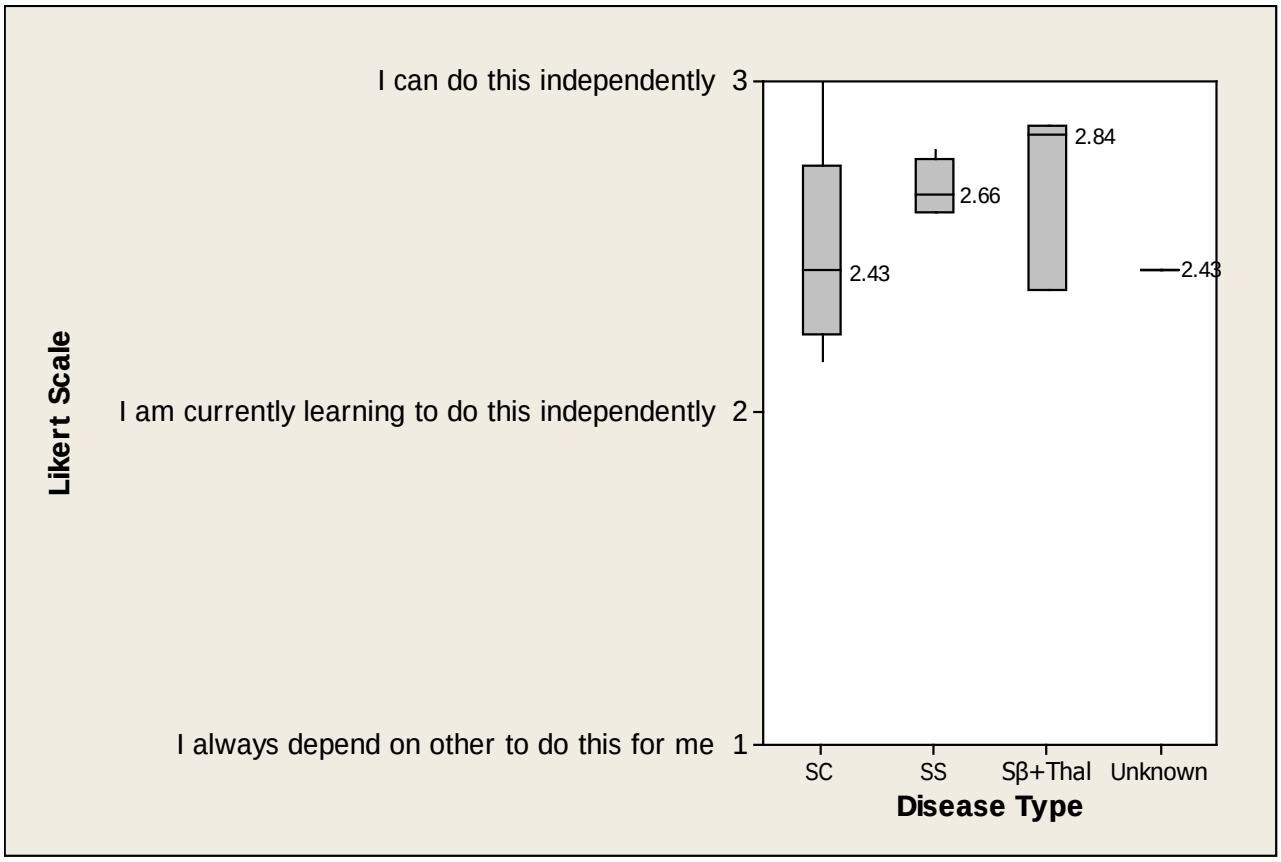

Figure 12. Comparison of Patients' Independence/Readiness by Disease Type

5.0 DISCUSSION

5.1 SPECIFIC AIM 1

This project sought to elicit the perspectives of sickle cell patients' in a pediatric setting regarding transition, in order to determine their expectations and concerns about the transition process. In general, a major barrier to transition is a lack of transitional support. (Bryant & Walsh, 2009). Additionally, this issue has been frequently emphasized in research regarding transition in the sickle cell population. Previous studies have shown that a major barrier to successful transition for these patients is a lack of adult hematology providers with expertise or interest in sickle cell disease (Andemariam, *et al*., 2014; Sobota, *et al*., 2011). Despite the fact that there is a dedicated adult hematology program that collaborates with the pediatric sickle cell clinic at CHP, a major worry expressed by the patients in this study was a concern about the level of support that they would receive from adult providers. These concerns are founded, given that there are significant gaps between the cultures of care in pediatric and adult care. A few of these differences noted in the literature include the amount of time patients may have to wait to be seen by an adult provider and the length of clinic appointments, as well as potential under treatment of symptoms when information regarding their pain management plan is not available. This is due to the fact that patients are often perceived as drug seeking when presenting for treatment of pain crises (Cerns, *et al*., 2013; Wilson & Nelson, 2014). The

implementation of various peer-based activities is one strategy that could be used to address this barrier. The utilization of a buddy system in which patients preparing to transition are paired with someone who has already gone through the transition process could potentially alleviate some of these concerns and expectations. By being able to hear firsthand accounts of patients' experiences with the adult care system, individuals would be able to find out whether their fears are truly justified or not. Additionally, group meetings in the pediatric setting in which individuals can freely express their concerns with other patients preparing to transition may also prove helpful. Moreover, these group meetings do not necessarily have to be something that is organized by the clinic itself. For example, the CHP Sickle Cell Clinic has a partnership with a local community-based organization, the Children's Sickle Cell Foundation (CSCF). At specific times throughout the year, CSCF holds a month-long transition program for young adults with sickle cell disease. This program is an additional source of support and educational opportunities for continued development. Providing patients with information about this program is another resource that could be offered by the CHP Sickle Cell Clinic.

The research that has evaluated the limited transition programs that currently exist has stressed the importance of implementing a comprehensive plan to successfully transition patients. This includes the development of formal transition programs by individual institutions, and the establishment of transition policy that defines the expected age of transfer, and the responsibilities for all stake holders during transfer (de Montalembert, *et al*., 2014). Additionally, the importance of contact with adult providers and health care centers prior to transition has been repeatedly mentioned. The results of this study further support these findings given that the main concerns expressed by patients in this study regarding their health care management were leaving their current doctor/health care team and establishing new

relationships with adult providers. These individuals were fearful of leaving the providers that they had grown accustomed too, and who they felt understood them. This has been reported in the transition literature (Cerns, *et al.*, 2013, Jordan & Coates, 2013; Smith, *et al.*, 2011). Although some patients were excited about the opportunity to work with new providers, the majority were hesitant about forming new relationships with these providers. Suggestions to address this barrier mentioned in the literature include better communication between pediatric and adult providers (van Staa, *et al.* 2011), and having patients meet with adult providers prior to transition (Hankins, et al., 2012; Jordan & Coates, 2013; McPherson, *et al.*, 2009; Tuchman, et al., 2008) . Therefore, a recommendation for the CHP transition program based on the findings of this study would be to take full advantage of the collaborative relationship that exists between the pediatric and adult hematology programs within the UPMC health care system. This would consist of taking such measures as increased communication between pediatric and adult health care providers, and creating opportunities for patients to meet the new health care team and/or visit the adult-oriented health care facility prior to transitioning.

Lastly, the aspect of transition that most of the patients appeared to be looking forward to was gaining independence and transitioning into adulthood. Similar findings have been documented in the literature; while patients are apprehensive about making the transition to adult care, they are excited for this milestone in their personal development (Doulton, 2010). Additionally, research has found that as the patient becomes more familiar with adult providers and are recognized as an adult, they often grow to find acceptance with transitioning (Bryant, *et al.*, 2011; Tuchman, et al., 2008).

5.2 SPECIFIC AIM 2

Factors such as, self-reliance, the patient's understanding of the disease, educational and socio-economic levels, and disease severity and complications can impact successful transition (Sobota, *et al*., 2014). This study aimed to assess patients' readiness for transition by eliciting their level of independence with regards to their perceptions of their knowledge and skills that are crucial for transition. This information is important to gather and assess given that is has been proposed that the greatest barrier to transition may be insufficient knowledge regarding the process (McPherson, *et al.*, 2009). Subsequently, understanding patients' perceptions of their level of knowledge, as well as their actual knowledge base, is an essential component of successful transition to adult-oriented care given that it influences anticipatory guidance.

In this study, patients appeared to have similar levels of independence in skills pertaining to "Knowledge of Health Issues/Diagnosis, "Being Prepared", and "Taking Charge". These similarities in levels of independence could be an artifact of the limited number of patients that were surveyed, and analysis of a larger sample of patients may potentially reveal distinct variations between these skill sets. However, despite the small sample size, there was still a significantly lower level of independence in skills related to "Preparing for Legal/Other Issues After Age 18". This difference may be due to the fact that in the clinical setting, health care professionals more often work to address issues pertaining to the actual medical condition (i.e. symptoms, appointments, and medications), rather than legal issues. Nonetheless, the literature has shown that it is vital that providers ensure that patients and families are prepared for, and understand the legal issues associated with the transition into adulthood. Youth with special health care needs are particularly affected by legal changes such as support-service or program eligibility changes, consent and confidentiality provisions, and issues related to insurance

coverage and payment for health care services (American Academy of Pediatrics, *et al*., 2011). In turn, a recommendation for the CHP Sickle Cell transition program would be to provide patients with resources regarding legal/other issues after age 18, whether it be in the form of a hard copy of written materials, web-based materials, and/or an educational discussion with a social worker.

In addition, previous studies on transition have consistently called for the utilization of patient readiness assessment tools (McPherson, *et al.*, 2009; Sobota, *et al*., 2014), followed by reassessment of the general transition plan in order to tailor to the specific needs of the patient and family. Therefore, transition should be viewed as a continuum, and the process should be started early in order to ensure adequate time for patients to reach an appropriate level of readiness. In turn, continued monitoring and addressing of items in which the patient states he/she needs assistance with to achieve independence will be essential in the transition process of the patients involved in this study.

Lastly, overall level of independence/readiness was found to not be influenced by patients' age, gender, or disease type in this study. This finding may have also been subject to sample size, given that previous research has shown that an older age at the start of transition negatively influenced transition success. Furthermore, patients with SC and $S\beta^+$ were shown to be more likely to unsuccessfully transition (Andemariam, et al., 2014). Given that age, gender, and disease type/severity did not influence the level of independence/readiness in our sample, there may be other factors that also play into determining the successfulness of the transition process. Moreover, results from this project as well as other studies have demonstrated that there are emotional and psychological factors that play into transition, and steps must be made to

also evaluate and address these issues (Smith, *et al.*, 2011; Tuchman, et al., 2008; Wills, *et al.*, 2010).

5.3 STUDY LIMITATIONS

One limitation of this study is its generalizability. The results of this study are based on a small sample of patients. The limited number of patients makes it difficult to generalize the data and findings to the entire CHP patient population. This is especially true when looking at the demographic data and the skill set analysis done based on the demographic characteristics. Furthermore, due to small sample size, outliers may significantly skew the results.

Another limitation is that the data collected and the analysis that could be performed from the data is dependent upon what patients chose to report. The degree to which patients' perspectives on transition could be assessed was reliant upon how much detail they put into the open-ended responses. Moreover, there were several surveys in which patients left one or more questions unanswered. This makes it difficult to fully interpret the data that was collected. Conversely, use of an adapted version of the customizable, standardized readiness assessment tool provided by Got Transition was a strength in regards to the data that was collected and analyzed.

Lastly, the patients involved in this study attend a uniquely designed sickle cell specialty clinic, which also has a close relationship with an adult hematology program. The perspectives and experiences of the patients in this study may not be an accurate representation of those held by all sickle cell patients in a pediatric setting preparing to transition to adult oriented care.

5.4 FUTURE CONSIDERATIONS

The results of this study provided some initial information regarding expectations and concerns of sickle patients regarding transition, and the level of independence they possess for various skills necessary to transition to an adult setting. The findings suggest possible recommendations for strategies that can be utilized by the transition program at the CHP Sickle Cell Clinic to address the obstacles to transition identified in the study. Consequently, an important next step would be to directly ask patients what they would want provided to them by the transition program in order to facilitate a more successful transition process.

Further research into the effectiveness of early and ongoing preparation for adult transition is still warranted. The survey instrument utilized in this study will be used longitudinally by the CHP Sickle Cell Clinic, to determine what factors positively impact the process of transition from pediatric to adult sickle cell disease health care. Additionally, it will be important to study patients who do not make successful transitions to adult oriented care in order to further identify and address barriers to transition. The development of a standardized survey used nationwide would provide greater insight into how other sickle cell centers are conducting transitions for their patients, and would help overcome the limitations associated with small sample size.

6.0 CONCLUSIONS

In conclusion, this project successfully met its specific aims. Eliciting sickle cell patients' expectations and concerns regarding transition, as well as evaluating their perceptions of knowledge and skills, generated understanding about potential barriers to transition to adult care. This information can inform the development of a plan to address these obstacles and provide a more successful transition process. From a public health perspective, sickle cell patients are typically transferred to adult health care providers around age 18 to 20 years. However, patients in the age of transition are often at an increased risk for significant disease complications, and young adults (18–30 years) with sickle cell disease often demonstrate a high need for hospital-based care (DeBaun & Telfair, 2012; de Montalembert, *et al*., 2014). In fact, individuals in this age group typically have a greater number of emergency room visits, in-patients stays, and acute care visits than their adolescent (10-17 years) and older adult (31-45 years) peers (Lebensburger, *et al*., 2012). In turn, transition from pediatric to adult care providers is a crucial step in the care of individuals with sickle cell disease, and the need for a strong transition program is essential.

APPENDIX A: IRB Approval Letter

University of Pittsburgh
Institutional Review Board

3500 Fifth Avenue
Pittsburgh, PA 15213
(412) 383-1480
(412) 383-1508 (fax)
http://www.irb.pitt.edu

Memorandum

To: Debra Cohen MD
From: IRB Office
Date: 8/13/2014
IRB#: REN14080038 / PRO13030461
Subject: Transition Together, a study of pediatric patients with sickle cell disease as they transition to adult health care

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Your renewal for the above referenced research study has received expedited review and approval from the Institutional Review Board under:
45 CFR 46.110.(5)
45 CFR 46.110.(7)

Please note the following information:

Approval Date:	8/13/2014
Expiration Date:	9/9/2015

Please note that it is the investigator's responsibility to report to the IRB any unanticipated problems involving risks to subjects or others [see 45 CFR 46.103(b)(5) and 21 CFR 56.108(b)]. Refer to the IRB Policy and Procedure Manual regarding the reporting requirements for unanticipated problems which include, but are not limited to, adverse events. If you have any questions about this process, please contact the Adverse Events Coordinator at 412-383-1480.

The protocol and consent forms, along with a brief progress report must be resubmitted at least **one month** prior to the renewal date noted above as required by FWA00006790 (University of Pittsburgh), FWA00006735 (University of Pittsburgh Medical Center), FWA00000600 (Children's Hospital of Pittsburgh), FWA00003567 (Magee-Womens Health Corporation), FWA00003338 (University of Pittsburgh Medical Center Cancer Institute).

Please be advised that your research study may be audited periodically by the University of Pittsburgh Research Conduct and Compliance Office.

APPENDIX B: TRANSITION QUESTIONAIRRE (SECTION 2)

A FEW THOUGHTS ABOUT TRANSITION

What are the good things about transitioning?

What are the not so good things about transitioning?

What is the best thing that will happen to you when you transition?

What is the worst thing that you see happening to you when you transition?

Can you state at least one thing that you have done in the past 6 months to improve the transition process?

APPENDIX C: TRANSITION QUESTIONAIRRE (SECTION 4)

VISIT EVALUATION WITH SICKLE CELL TEAM (SCD SPECIFIC)

C.1 KNOWLEDGE OF HEALTH ISSUES/DIAGNOSIS

1. **I can explain the type of sickle cell disease that I have.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
2. **I understand my diagnosis and health history, and can explain it to others.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
3. **I know and can state my significant health problems caused by my sickle cell disease.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
4. **I know what my other non-sickle cell diagnoses are.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
5. **I can explain my health care needs and medical treatments to a new provider.**

- **I can do this independently**
- **I am currently learning to do this independently**
- **I always depend on others to do this for me**

6. **I can tell a new provider what medication I have been prescribed.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
7. **I can state the dose and how often I take the medication.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
8. **I know how to pick up my medicine from the pharmacy.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
9. **I can inform the provider what specialists I have seen.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
10. **I know when I last saw each specialist.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**

11. I know my hemoglobin and retic level baseline.

- **I can do this independently**
- **I am currently learning to do this independently**
- **I always depend on others to do this for me**

12. I know my pulse oximeter baseline number.

- **I can do this independently**
- **I am currently learning to do this independently**
- **I always depend on others to do this for me**

13. I know my symptoms that need quick medical attention.

- **I can do this independently**
- **I am currently learning to do this independently**
- **I always depend on others to do this for me**

14. I know what to do in case I have a medical emergency.

- **I can do this independently**
- **I am currently learning to do this independently**
- **I always depend on others to do this for me**

C.2 BEING PREPARED

1. I carry my health insurance card every day.

- **I can do this independently**
- **I am currently learning to do this independently**

- **I always depend on others to do this for me**

2. **I know what type of Medical Coverage I have (i.e. Medicaid, UPMC, Gateway, etc.) and I know the basic services that are covered.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
3. **I either carry or know where to get my important health information (i.e. medical summary, including medical diagnosis, list of medications, allergy info, doctor's numbers, drug store number, etc.).**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
4. **Do you have an adult Primary Care Physician (If you are over 18)?**
    - **Yes**
    - **No**

C.3 TAKING CHARGE

1. **I call and make my own doctor's appointments.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**

2. **I use a cell phone or calendar to remind me of appointments and refills.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
3. **I know I have an option to see my doctor by myself.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
4. **Before a doctor's appointment I prepare written questions to ask.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
5. **I track my own appointment and prescription refill expiration dates.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
6. **I can read a prescription label.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
7. **I call in my own prescription refills.**
    - **I can do this independently**
    - **I am currently learning to do this independently**

- **I always depend on others to do this for me**

8. **I can state where I typically experience pain during a pain episode.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
9. **I know how to manage my pain and diagnosis at home.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
10. **I know what medication helps to manage my pain.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
11. **I know how to take my medications to manage my pain.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
12. **I know what non-medication steps to take to help manage my pain.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
13. **I know stress reduction or relaxation techniques that help my pain.**
    - **I can do this independently**

- **I am currently learning to do this independently**
- **I always depend on others to do this for me**

C.4 PREPARING FOR LEGAL/OTHER ISSUES AFTER AGE 18

1. **I have a plan so I can keep my health care insurance or obtain medical coverage after I turn 18.**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
2. **I have a plan to develop a legal Power of Attorney for health care decisions in the event my health changes and I am unable to make decisions for myself. (Everyone in the family should have one!)**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
3. **Do you know of resources that can help you find adult services (job support, transportation, assistive technology, etc.)?**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
4. **Do you know how your condition may affect your job choices?**

- **I can do this independently**
- **I am currently learning to do this independently**
- **I always depend on others to do this for me**

5. **Do you know what benefits you might qualify for (SSI, Community Based Services etc.)?**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
6. **Do you know your options for housing as an adult (on your own, group home)?**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**
7. **Do you know how to manage money and pay your bills?**
    - **I can do this independently**
    - **I am currently learning to do this independently**
    - **I always depend on others to do this for me**

BIBLIOGRAPHY

American Academy of Pediatrics; American Academy of Family Physicians; American College of Physicians-American Society of Internal Medicine. (2002) A consensus statement on health care transitions for young adults with special health care needs. *Pediatrics*. 110(6 Pt 2):1304-6.

American Academy of Pediatrics; American Academy of Family Physicians; American College of Physicians; Transitions Clinical Report Authoring Group, Cooley, W.C., Sagerman, P.J. (2011) Supporting the health care transition from adolescence to adulthood in the medical home. *Pediatrics*. 128(1):182-200.

Andemariam, B., Owarish-Gross, J., Grady, J., Boruchovm D., Thrallm,, R.S., & Hagstrom, J.N. (2014) Identification of risk factors for an unsuccessful transition from pediatric to adult sickle cell disease care. *Pediatr Blood Cancer*. 61(4):697-701.

Bender, M.A., & Douthitt Seibel, G. Sickle Cell Disease. (2003). In *GeneReviews® [Internet]*. Edited by Pagon, R.A., Adam, M.P., Ardinger, H.H., *et al.*Seattle (WA): University of Washington, Seattle; 1993-2015. [Updated 2014 Oct 23].

Braun V., & Clarke V. (2006) Using thematic analysis in psychology. *Qualitative Research in Psychology*. 3(2):77–101.

Bryant, R. & Walsh, T. (2009) Transition of the chronically ill youth with hemoglobinopathy to adult health care: an integrative review of the literature. *J Pediatr Health Care*. 23(1):37-48.

Bryant, R., Young, A., Cesario, S., & Binder, B. (2011) Transition of chronically ill youth to adult health care: experience of youth with hemoglobinopathy. *J Pediatr Health Care*. 25(5):275-83.

Clarke, G.M., & Higgins, T.N. (2000) Laboratory investigation of hemoglobinopathies and thalassemias: review and update. *Clin Chem*. 46(8 Pt 2):1284-90.

Cerns, S., McCracken, C., & Rich, C. (2013) Optimizing adolescent transition to adult care for sickle cell disease. *Medsurg Nurs*. 22(4):255-7.

Cooley, W. C., & Sagerman, P. J. (2011). Supporting the health care transition from adolescence to adulthood in the medical home. *Pediatrics*. 128(1): 182-200.

DeBaun, M.R. & Telfair, J. (2012) Transition and sickle cell disease. *Pediatrics*. 130(5):926-35.

de Montalembert, M., Guitton, C.; French Reference Centre for Sickle Cell Disease. (2014) Transition from paediatric to adult care for patients with sickle cell disease. *Br J Haematol*. 164(5):630-5.

Doulton, D.M. (2010) From cradle to commencement: transitioning pediatric sickle cell disease patients to adult providers. *J Pediatr Oncol Nurs*. 27(2):119-23.

Hankins, J.S. *et al*. (2012) A transition pilot program for adolescents with sickle cell disease. *J Pediatr Health Care*. 26(6):e45-9.

Jordan, L., Swerdlow, P., & Coates, T.D. (2013) Systematic review of transition from adolescent to adult care in patients with sickle cell disease. *J Pediatr Hematol Oncol*. 35(3):165-9.

Kanter, J. & Kruse-Jarres, R. (2013) Management of sickle cell disease from childhood through adulthood. *Blood Rev*. 27(6):279-87.

Lebensburger, J.D., Bemrich-Stolz, C.J., & Howard, T.H. (2012) Barriers in transition from pediatrics to adult medicine in sickle cell anemia. *J Blood Med*. 3:105-12.

McManus, M. A., Fox, H., O'Connor, K., Chapman, T., & MacKinnon, J. (2008). Pediatric perspectives and practices on transitioning adolescents with special needs to adult health care. Washington DC: National Alliance to Advance Adolescent Health.

McPheeters, M., Davis, A.M., Taylor, J.L., Brown, R.F., Potter, S.A., & Epstein, R.A. (2014) Transition Care for Children With Special Health Needs [Internet]. Rockville (MD): Agency for Healthcare Research and Quality (US). Available from http://www.ncbi.nlm.nih.gov/books/NBK222123/.

McPherson, M., Thaniel, L., & Minniti, C.P. (2009) Transition of patients with sickle cell disease from pediatric to adult care: Assessing patient readiness. *Pediatr Blood Cancer*. 52(7):838-41.

Meier, E.R., & Miller, J.L. (2012) Sickle cell disease in children. *Drugs*. 72(7):895-906.

Molter, B.L., & Abrahamson, K. (2014) Self-Efficacy, Transition, and Patient Outcomes in the Sickle Cell Disease Population. *Pain Manag Nurs*. pii: S1524-9042(14)00095-2.

National Health Care Transition Center. (2015) Got transition? Available from http://www.gottransition.org.

Porter, J.S., Matthews, C.S., Carroll, Y.M., Anderson, S.M., Smeltzer, M.P., & Hankins, J.S. (2014) Genetic education and sickle cell disease: feasibility and efficacy of a program tailored to adolescents. *J Pediatr Hematol Oncol*. 36(7):572-7.

Quinn C.T. (2013) Sickle cell disease in childhood: from newborn screening through transition to adult medical care. *Pediatr Clin North Am*. 60(6):1363-81.

Rees, D.C., Williams, T.N., & Gladwin, M.T. (2010) Sickle-cell disease. *Lancet*. 376(9757):2018-31.

Smith, G.M., Lewis, V.R., Whitworth, E., Gold, D.T., & Thornburg, C.D. (2011) Growing up with sickle cell disease: a pilot study of a transition program for adolescents with sickle cell disease. *J Pediatr Hematol Oncol*. 33(5):379-82.

Sobota, A. *et al*. (2014) Self-reported transition readiness among young adults with sickle cell disease. *J Pediatr Hematol Oncol*. 36(5):389-94.

Sobota, A., Neufeld, E.J., Sprinz, P., & Heeney, M.M. (2011) Transition from pediatric to adult care for sickle cell disease: results of a survey of pediatric providers. *Am J Hematol*. 86(6):512-5.

Treadwell, M., Telfair, J., Gibson, R.W., Johnson, S., & Osunkwo, I. (2011) Transition from pediatric to adult care in sickle cell disease: establishing evidence-based practice and directions for research. *Am J Hematol*. 86(1):116-20.

Tuchman, L.K., Slap, G.B., & Britto, M.T. (2008) Transition to adult care: experiences and expectations of adolescents with a chronic illness. *Child Care Health Dev*. 34(5):557-63.

van Staa, A.L., Jedeloo, S., van Meeteren, J., & Latour, J.M. (2011) Crossing the transition chasm: experiences and recommendations for improving transitional care of young adults, parents and providers. *Child Care Health Dev*. 37(6):821-32.

Wills, K.E. *et al*. (2010) Transition planning for youth with sickle cell disease: embedding neuropsychological assessment into comprehensive care. *Pediatrics*. 126 Suppl 3:S151-9.

Wilson, B.H., & Nelson, J. (2014) Sickle Cell Disease Pain Management in Adolescents: A Literature Review. *Pain Manag Nurs*. pii: S1524-9042(14)00102-7.

Yawn, B.P. et al. (2014) Management of sickle cell disease: summary of the 2014 evidence-based report by expert panel members. *JAMA*. 312(10):1033-48.