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I, Ilana Barach, hereby submit this original work as part of the requirements for the degree of Master of Arts in Psychology.

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Disease Management and Psychosocial and Health Outcomes in Pediatric Sickle Cell Disease

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Disease Management and Psychosocial and Health Outcomes in Pediatric Sickle Cell Disease

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By
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Abstract

Introduction

As youth with a chronic illness transition through adolescence they must negotiate typical developmental milestones in addition to learning to manage their disease. An important aspect of this process involves youth taking on more responsibility for disease management with greater age and maturity. The purpose of this study is to increase our understanding of disease management responsibility in pediatric sickle cell disease (SCD) and the relationship between disease management responsibility and psychosocial and health outcomes. Specifically, the study aims to: 1) examine the relationships between youth and caregiver responsibility for disease management and age 2) examine the relationships between youth and caregiver responsibility for disease management and youth functioning (as measured by the CBCL/YSR) and family functioning (as measured by the FAD); and 3) examine the relationships between youth and caregiver responsibility for disease management and correlates of disease severity (urgent healthcare utilization and hemoglobin type).

Methods

Participants and procedures. Youth with SCD, ages 8 to 21, who are receiving care at the Cincinnati Comprehensive Sickle Cell Clinic, and their caregivers, completed questionnaires at the annual Sickle Cell Research and Education Day event held at Cincinnati Children’s Hospital, or during regularly scheduled clinic visits.

Procedure. Questionnaires included: 1) a demographics and medical history form; 2) The Responsibility Scale for Pediatric SCD-Revised; 3) The Family Assessment Device (FAD) General Functioning Subscale; and 4) Child Behavior Checklist (CBCL) for Ages 6 to 18 parent report and youth self-report. Additionally, participants’ electronic medical records were reviewed.
Results

Older youth age was moderately correlated with caregiver ($r = 0.52, p = .00$) and youth report ($r = 0.64, p = .00$) of greater youth responsibility for disease management. Caregivers tended to have more responsibility for disease management than youth, especially for tasks involving navigation of the health care and school systems, and youth reported sharing responsibility for tasks having to do with symptom self-monitoring and decision-making. Higher levels of youth Externalizing Behaviors ($r = 0.36, p = .04$) and Withdrawn/Depressed symptoms ($r = 0.28, p = .05$) were associated with less youth responsibility for disease management. Family functioning and hemoglobin type were not associated with responsibility. Increased levels of self-reported responsibility by youth was associated with higher numbers of ER visits ($r = 0.34, p = .01$).

Conclusions/Implications

The present findings are significant given the need to ensure that youth are able to manage their disease independently over time and into adulthood. Future research should continue to examine management of sickle cell disease during transitional periods including adolescence. The ultimate goal is to develop interventions to ensure that caregivers and youth are optimally involved in disease management and that tasks are transferred to youth over time, while also promoting adherence with the treatment regimen.
Acknowledgements

I have been blessed with a team working behind the scenes to support me in my graduate study and most recently with my thesis. There are countless souls who have helped me get this far, much too numerous to list; below is my attempt to acknowledge a few.

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CHAPTER 1

Introduction

Youth with sickle cell disease (SCD) experience chronic symptoms, which may result in a host of physical and social-emotional complications, such as painful vaso-occlusive episodes, infection, stroke, and organ damage (NIH, 2002). As a result, the treatment regimen for these youth is often very complex, requiring routine clinic visits and immunizations as well as lifestyle changes (e.g. sufficient hydration and rest; NIH, 2002). Treatment also commonly includes medications (e.g. Hydroxyurea, and chelation medications) and medical procedures such as blood transfusions (NIH, 2002). Unexpected symptoms and complications, such as vaso-occlusive pain episodes and life-threatening infections may lead to ER visits and hospitalizations (NIH, 2002). Effective management of SCD is labor intensive and requires close monitoring by parents and medical professionals (Palermo & Chambers, 2005; Wallander, Varni, Babani, Banis, & Wilcox, 1989).

According to the literature, disease management tasks should involve both youth and caregivers, and responsibilities placed upon youth should be appropriate to their developmental level (Palermo & Chambers, 2005; Wallander, et al., 1989). Responsibility for disease management is known to increase during adolescence, while medical adherence is known to decrease at this time (Drotar & Ievers, 1994; Naar-King et al., 2009; Orrell-Valente, Jarlsberg, Hill, & Cabana, 2008). Yet, if adolescents with SCD do not properly manage their illness, they increase their risk for medical complications and poor health outcomes in the future.

For youth with a chronic illness, transition through adolescence may be especially difficult, as they must negotiate typical developmental milestones while managing their disease. Disease management has been associated with psychosocial and health outcomes (Helgeson,
Reynolds, Siminerio, Escobar, & Becker, 2008; Palmer et al., 2009), as well as adherence to medical regimens (Anderson et al., 2002). How parents and children manage responsibilities for disease management tasks, particularly during adolescence, has been studied in other diseases, such as type 1 diabetes, asthma, cystic fibrosis, and HIV (Modi, Marcie, Slater, Drotar, & Quittner, 2008; Naar-King, et al., 2009; Orrell-Valente, et al., 2008; Pai et al., 2010; Palmer et al., 2004); however, very few studies have examined this issue in pediatric SCD (Alvarez et al., 2009; Beyer & Simmons, 2004; Oliver-Carpenter, Barach, Crosby, Valenzuela, & Mitchell, 2011; Treadwell et al., 2005; Treadwell & Weissman, 2001). These published SCD studies have primarily focused on the management of chelation therapy and pain.

There are a number of published validated, disease-specific measures to assess the division of caregiver and youth disease management responsibility (Anderson, Auslander, Jung, Miller, & Santiago, 1990; Greenley, Doughty, Stephens, & Kugathasan, 2010; McQuaid, Penza-Clyve, Nassau, & Fritz, 2001; Pai, et al., 2010). The existence of these measures has promoted a better understanding of this topic, especially in pediatric diabetes and asthma, through creating a standardized approach to measuring how caregivers and youth collaborate and coordinate disease management tasks. Prior to this study, a measure of disease management responsibility specific to pediatric SCD was piloted (Oliver-Carpenter, et al., 2011). The measure was adapted from the Diabetes Family Responsibility Questionnaire (Anderson, et al., 1990) and the Asthma Responsibility Questionnaire (McQuaid, et al., 2001).

The piloted version of the measure was then revised to address issues that surfaced in the pilot study, such as the need for a simpler format to promote ease of administration. The measure was also revised to better align with validated responsibility measures in diabetes and asthma. Although the revised measure has not yet been validated, preliminary analyses suggest good
reliability. The current study extends this line of research to examine the association between caregiver and youth disease management responsibility and developmental, psychosocial, and health correlates using this revised version of the disease responsibility measure.

**Literature Review**

Effective disease management properly controls symptoms and disease pathophysiology, thereby promoting good health and quality of life, by reducing disease symptoms and complications. Good adherence to one’s medical regimen is an important aspect of disease management; however, disease management also requires that the patient frequently self-monitor and adjust behavior accordingly (Clark, 2003). Management of a pediatric disease is a complex and dynamic phenomenon that involves partnerships between the pediatric patient, their caregivers and other family members, and their medical team (Buchbinder, 2009; De Civita & Dobkin, 2004). Ideally, caregivers of youth with chronic conditions (and later the youth themselves) become competent at managing their illness, but it is common for youth and their families to struggle with disease management, which increases the risk unexpected symptoms and complications. Some of the factors important for understanding the management of pediatric conditions are developmental factors, such as age, family functioning, youth psychosocial functioning, illness severity, and health care utilization.

**Age, Development, and Disease Management**

Disease management occurs as children and adolescents observe caregiver management behaviors, engage in patient-provider interactions, mature, and experience other social and developmental processes (Orrell-Valente & Cabana, 2008). Young children often do not have the developmental capabilities to manage their illness on their own, thereby requiring that their caregiver take primary responsibility (Golden, 1999; Schilling, Knafl, & Grey, 2006). Although
caregivers may engage in most illness management tasks for their young child, Mitchell and colleagues (2007), have suggested that children with a chronic illness play an important role in the management of their illness, even at a young age; therefore management needs to be viewed as a partnership between youth and their caregivers (Mitchell et al., 2007). Additionally, partnerships between caregivers, their youth, other family members, and the health care team are crucial in promoting effective disease management (Buchbinder, 2009; De Civita & Dobkin, 2004).

There is consensus in the literature that youth with a chronic illness take on increasing levels of responsibility for the management of their illness as they get older (Drotar & Ievers, 1994; Naar-King, et al., 2009; Orrell-Valente, et al., 2008), likely using some of the management strategies and patterns that they observe their caregivers using. Although, imprecise, age is commonly used as a proxy for developmental factors such as autonomy, self-efficacy, and competence (De Civita & Dobkin, 2004). There is evidence, though, that developmental factors such as disease management self-efficacy and autonomy may be more important than age in determining youth readiness to take on more responsibility for managing their illness (Holmes et al., 2006; Palmer, et al., 2009). Findings in the literature are varied regarding the age at which youth become the primary managers of their illness, perhaps due to differences in developmental factors, as well as differences in disease characteristics. For example, although some studies found that adolescents generally manage most of their illness care by 15 to 17 years of age (Orrell-Valente, et al., 2008; Schilling, et al., 2006), other studies suggest that adolescents may not become primary managers until later (Marhefka et al., 2008).

Regardless of the exact age of responsibility transfer, research across chronic illness conditions has found that parental involvement and monitoring to ensure disease management
promotes improved adherence and positive health outcomes (Berg et al., 2008; Berg et al., 2011; Ellis et al., 2007; Helgeson, et al., 2008; Modi, et al., 2008; Naar-King, et al., 2009; Palmer, et al., 2004; Palmer et al., 2011; Reed-Knight, Lewis, & Blount, 2011; Vesco et al., 2010). This seems to be the case even after the adolescent becomes the primary disease manager.

It is recommended that caregivers promote youth competence in disease management through a gradual process over the course of many years to prepare them for independence and transitioning to adult health care (Reiss & Gibson, 2002). This process is consistent with the process recommended for encouraging behavioral autonomy, whereby caregivers gradually give up small amounts of responsibility and decision-making authority to their adolescents over a period of years (Holmbeck, 1996; Spear & Kulbok, 2004). There is evidence that this shift in responsibility to the adolescent is imprecise and not necessarily linear (Pai et al., 2011).

While a great deal is known about how age relates to disease management responsibilities in a variety of pediatric chronic illnesses, studies in pediatric SCD have not dealt with age and developmental factors and their role in disease management responsibility. Therefore, there is a need for research specific to pediatric SCD, examining age as a factor in the transfer of disease management responsibility from caregiver to youth.

**Disease Management and Family Functioning**

Conducting studies in the family context is especially important in pediatric SCD given the cultural importance of family networks for African Americans (Hattery & Smith, 2007). Results from one study indicate that families of youth with SCD may be similar to families of normative African American youth on all family functioning subscales except control; the SCD-affected families had more set rules and procedures than did normative African American families (Barakat et al., 2007).
The pediatric SCD literature suggests that family dysfunction is associated with poor adherence and negative health outcomes. Barakat, Smith-Whitley, and Ohene-Frempong (2002) found that effective problem solving and behavior control in families with SCD were associated with good adherence. On the other hand, family functioning composite scores were not associated with health outcome variables in two pediatric SCD studies (Barakat, et al., 2007; Mitchell, et al., 2007). However, when families were divided into three groups based on youth and caregiver concordance of report of family functioning, families where both youth and caregiver reported family dysfunction had worse health outcomes than did families where caregiver and youth both reported good family functioning and families where caregiver and youth report of family functioning was discordant (Barakat, et al., 2007).

These findings suggest that family functioning is a crucial factor in the management of pediatric SCD. However, most of the literature addressing family functioning in pediatric SCD has focused on how family functioning is associated with child psychosocial outcomes, and very few studies have addressed family functioning in pediatric SCD and its association with disease management. Given the dearth of literature addressing family functioning and disease management specific to pediatric SCD, the general pediatric literature helps to provide a theoretical context; however caution is warranted, as the pediatric literature on family functioning may not be fully generalizable to pediatric SCD.

The pediatric literature generally suggests that disease management is more likely to be consistent and effective in a stable and supportive family environment. Family functioning has been shown to be associated with health outcomes (Barakat, et al., 2007; Leonard, Jang, Savik, & Plumbo, 2005), and it has been proposed that this association may be partially mediated by management behaviors (Kaugars, Klinnert, & Bender, 2004). This model is supported by the
consistent finding that family characteristics are associated with adherence to medical regimens. Family factors that indicate healthy family functioning, such as cohesion, warmth, and effective communication have been associated with good adherence (Cohen, Lumley, Naar-King, Partridge, & Cakan, 2004; DeLambo, Ievers-Landis, Drotar, & Quittner, 2004; Weinstein & Faust, 1997), while factors suggestive of family dysfunction, such as family conflict, disengagement, and rejection have been associated with poor adherence (Chen, Bloomberg, Fisher, & Strunk, 2003; Kaugars, et al., 2004; Lewin et al., 2006; Wamboldt & Wamboldt, 2000; Wiebe et al., 2005). This literature also supports that the relationship between family characteristics and disease management is likely transactional, rather than unidirectional (Kaugars, et al., 2004). This means that family dysfunction, difficulties in disease management, and resulting negative health outcomes may result in a vicious, self-sustaining cycle.

There is evidence that the association of parent involvement and positive health outcomes may only occur in the context of a healthy caregiver-child relationship, characterized by warmth, acceptance and collaboration (Wysocki et al., 2009). This may be especially important as youth get older because parent involvement in a supportive family context is less likely viewed as intrusive by adolescents as they desire increasing levels of autonomy. The family system may be increasingly stressed, as roles and responsibilities for disease management shift during adolescence. At this time, families require stability, organization, flexibility, and effective communication to adjust management responsibilities according to the youth’s changing developmental capabilities (Kaugars, et al., 2004).

While the pediatric literature provides a theoretical context for understanding family functioning and disease management, additional research in pediatric SCD is needed to better understand the unique aspects of the disease on family systems. Given that most individuals with
SCD in the U.S. are African American, factors specific to African American families are likely to play an important and dynamic role in family functioning for youth with SCD.

**Disease Management and Youth Psychosocial Functioning**

Youth psychosocial functioning is closely linked with disease management, as youth need good mental health in order to effectively manage their illness independently. Additionally, youth need to have a certain level of conscientiousness and attention to detail in order to adhere to their regimen. Therefore, it makes sense that the literature suggests that externalizing behavior is associated with lower levels of adherence and worse disease control (Bryden et al., 2001; Duke et al., 2008; Horton, Berg, Butner, & Wiebe, 2009) and fewer adherence behaviors (Holmes, et al., 2006). Difficulties in adherence and family disease management may mediate the association between youth externalizing symptoms and disease morbidity, as demonstrated in pediatric asthma, where management involving complex, coordinated actions was especially negatively affected by ADHD symptoms (McQuaid et al., 2008). Internalizing symptoms, such as depression have been associated with fewer self-management behaviors in pediatric type 1 diabetes (Holmes, et al., 2006; Law, Kelly, Huey, & Summerbell, 2002). Additional research suggests that self-efficacy is an important factor in disease management (Rhee, Belyea, Ciurzynski, & Brasch, 2009). Self-efficacy may play a role in the association between internalizing symptoms and disease management difficulties, given that negative thinking and low self-efficacy are correlates of depression (Beck, 2008; Muris, 2002).

**Parent and youth management responsibility.** While internalizing and externalizing symptoms appear to be associated with worse adherence, there is little consensus in the pediatric literature over the association of responsibility for disease management and youth psychosocial adjustment, and research is limited. One study failed to confirm a relationship between
responsibility for disease management and psychosocial adjustment (Holmes, et al., 2006), while two studies found a relationship, indicating that parental supervision is associated with better quality of life (Graue, Wentzel-Larsen, Hanestad, & Sovik, 2005; Wiebe, et al., 2005).

Youth psychosocial functioning and disease management in SCD. Youth with SCD, as youth with any chronic illness, are at risk for low mood, low self-esteem, and feelings of hopelessness (Anie, 2005; Barbarin, 1999; Trzepacz, Vannatta, Gerhardt, Ramey, & Noll, 2004). Given that the stresses of SCD may result in psychological symptoms, it is important to examine the association between psychosocial adjustment and disease management. The SCD literature has found that psychosocial factors such as coping are associated with health care utilization and pain (Fletcher, 2000; Gil et al., 2003; Gil et al., 2004; Mitchell, et al., 2007); however, there is very little disease-specific research on the relationship between psychosocial adjustment and disease management, and the findings are inconsistent. Surprisingly, adherence to medical regimen in pediatric SCD has been associated with poorer quality of life (Barakat, Lutz, Smith-Whitley, & Ohene-Frempong, 2005), but in another study, adherence was associated with better quality of life, and barriers to adherence mediated this relationship (Fisak, Belkin, von Lehe, & Bansal, 2012). Finally, a third study did not find any association between psychosocial functioning and adherence, although the correlation between behavioral problems and adherence difficulties had a trend towards significance (Barakat, Smith-Whitley, & Ohene-Frempong, 2002). The available SCD-specific research has inconsistent findings regarding the association of psychosocial adjustment and disease management. There is a need for research examining disease management generally, and also whether who takes responsibility for management plays a role in this association.

Disease Management and Healthcare Utilization/Illness Severity in SCD
Hemoglobin type is commonly used as a proxy of illness severity in the SCD literature (Brown, Connelly, Rittle, & Clouse, 2006; Casey, Brown, & Bakeman, 2000; Lutz, Barakat, Smith-Whitley, & Ohene-Frempong, 2004). Hemoglobin SS is generally associated with the most severe disease trajectory (Al-Haggar, Al-Marsafawy, Abdel-Razek, Al-Baz, & Mostafa, 2006; Ashley-Koch, Yang, & Olney, 2000) and highest number of ER visits and hospitalizations (Anie, Steptoe, Ball, Dick, & Smalling, 2002; Rogovik, Li, Kirby, Friedman, & Goldman, 2009), although there is a great deal of heterogeneity of disease course even within respective hemoglobin types (Ashley-Koch, et al., 2000).

Across the pediatric SCD literature there is a lack of consistency for determining disease severity, although studies have provided several methods to predict later disease severity in infants and young children with SCD (Day, 2004; Miller et al., 2000). A recent publication piloted an index of disease severity among children ages 2 through 18 using criteria such as pain crises, acute chest syndrome, cerebrovascular infarcts, and organ damage (van den Tweel, van der Lee, Heijboer, Peters, & Fijnvandraat, 2010). Disease severity as measured by this index was moderately associated with number and length of inpatient stays as well as hemoglobin type (van den Tweel, et al., 2010). The SCD literature indicates that hemoglobin type and urgent health care utilization (ER visits and hospitalizations) are correlates of disease severity, and they have been conceptualized in this manner in the present study.

The literature, though limited, supports that disease management is strongly associated with ER visits and hospitalizations, especially in asthma (Fiese & Wamboldt, 2003). Additionally, disease management interventions have been shown to decrease emergency health care utilization (Afifi, Morisky, Kominski, & Kotlerman, 2007). Despite the association between disease management and health care utilization, there is need for research focusing on youth and
parent dynamic interactions surrounding disease management (e.g. who is taking responsibility for disease management tasks) and its association with disease severity and health care utilization. No studies in pediatric SCD have addressed this topic, although one study in pediatric asthma found that youth with a high level of responsibility for disease management tended to engage in fewer management behaviors and were less likely to seek preventative care or urgent care for asthma symptoms (Bruzzese et al., 2012).

Disease Management Measures and Assessment

Scales that measure parent and youth responsibility for disease management tasks have been validated and are commonly used in pediatric diabetes (Anderson, et al., 1990) and asthma (McQuaid, et al., 2001). More recently, responsibility measures have been developed for other pediatric conditions, such as HIV (Naar-King, et al., 2009), inflammatory bowel disease (Greenley, et al., 2010), and pediatric transplant patients (Pai, et al., 2010). These more recent scales were based on the original Diabetes Family Responsibility Questionnaire (DFRQ; Anderson et al., 1990) and the Asthma Responsibility Questionnaire (ARQ; McQuaid et al., 2001). The utilization of validated measures such as the DFRQ (Anderson, et al., 1990) has resulted in a deeper understanding of how responsibility is slowly transferred from caregiver to youth over the course of adolescence. The literature suggests that continued caregiver involvement in the form of monitoring and support of disease management are crucial for promoting positive psychosocial outcomes such as fewer externalizing behaviors as well as increased adherence and better health outcomes.

Although there is a large amount of research on child and caregiver responsibility for disease management in some pediatric illnesses (such as diabetes, asthma, and cystic fibrosis), research testing standardized assessment and measures in pediatric SCD is sparse. Given that the
majority of individuals with SCD are of African descent or African American, cultural sensitivity factors must be considered. Additionally, every disease has individual characteristics that may differentially affect disease management, so there is a need for a disease-specific inquiry into self-management of pediatric SCD.

Some SCD studies have examined caregiver involvement without a standardized measure, and suggest that caregiver involvement in disease management is also important in promoting positive outcomes in children and adolescents with SCD (Alvarez, et al., 2009; Treadwell, et al., 2005; Treadwell & Weissman, 2001). However, further research is needed focusing on a variety of disease management tasks specific to SCD using a standardized measure. In order to promote better disease management and improve health outcomes, it is important for this research to examine the relationship between disease management and outcomes, such as family and youth psychosocial functioning, disease severity, and health care utilization. A better understanding of responsibility for disease management tasks and its relationship to psychosocial and health outcomes will aid in promoting smoother transitions through adolescence and better disease management and health outcomes.

**Theoretical Model**

Kaugars, Klinnert, and Bender (2004) describe a theoretical model for understanding family characteristics and asthma outcomes. The model posits that family characteristics predict asthma outcomes through both asthma management behaviors and physiological functioning (e.g. HPA-Axis). The present study uses this model to examine family characteristics in pediatric SCD (family functioning, youth psychosocial adjustment, and responsibility for disease management), and health correlates (healthcare utilization; See Figure 2). In the present study, health care utilization is conceptualized as a correlate of disease severity and outcomes, rather
than an outcome itself, as the study’s cross sectional design does not allow for causal predictions. Additionally, Kaugars, Klinnert, and Bender (2004) do state that the model associations may be bidirectional. Nonetheless, the use of this model may aid in conceptualizing the variables examined in this study and provides direction for future research examining not just family characteristics, but also management behaviors such as adherence.

<table>
<thead>
<tr>
<th>Family Characteristics</th>
<th>Disease Outcomes</th>
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<td>- Responsibility for disease management</td>
<td>- Health care utilization</td>
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<tr>
<td>- Family functioning</td>
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<td>- Youth psychosocial adjustment</td>
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*Figure 1. Model of factors that contribute to disease outcomes. The present study uses this model to conceptualize how responsibility for disease management and other family characteristics are related to health care utilization (ER visits and hospitalizations). Adapted from “Family Influences on Pediatric Asthma” by A. S. Kaugars, M. D. Klinnert, and B. G. Bender, 2004, *Journal of Pediatric Psychology*, 29, p. 476. Copyright 2004 by the Society of Pediatric Psychology.*

**Aims and Hypotheses**

The purpose of this study is to increase our understanding of disease management responsibility in pediatric SCD and the relationship between disease management responsibility and psychosocial and health outcomes. Specifically, the study aims to: 1) examine the relationship between youth and caregiver responsibility for disease management and age; 2) examine the relationships between youth and caregiver responsibility for disease management and youth psychosocial functioning and family functioning; and 3) examine the relationships between youth and caregiver responsibility for disease management and disease severity (hemoglobin type) and urgent health care utilization (hospitalizations and ER visits).
**Hypothesis 1: Age and responsibility for disease management.** It is expected that older age will be associated with greater youth responsibility, as has been found in the literature for other pediatric illnesses (e.g. Naar-King et al., 2009; Orrell-Valente et al., 2008).

**Hypothesis 2: Family and youth functioning and responsibility for disease management.** We predict that worse family functioning will be associated with greater youth responsibility given literature supporting the impact of family-related variables on health outcomes. On the other hand, we predict that worse youth psychosocial functioning will be associated with greater caregiver responsibility because youth with lower psychosocial functioning may be less engaged and competent in managing their disease.

**Hypothesis 3: Disease severity and responsibility for disease management.** It is expected that worse disease severity and greater use of urgent health care will be associated with greater caregiver responsibility. This is predicted because acute illness may compromise self-care.
CHAPTER 2

Method

Participants

A convenience sample of 64 youth in a Midwestern city with SCD between the ages of 8 and 21 and their caregivers participated in this study. Youth were eligible if they had a diagnosis of SCD and were followed through the sponsoring institution’s pediatric sickle cell clinic, or had transferred to the adult sickle cell clinic at a collaborating institution ($N = 2$). Eligible participants were identified by their sickle cell care team. Letters signed by the Sickle Cell Center Director and Principal Investigator were sent to eligible families explaining the purpose of the study and inviting them to participate. The research protocol was approved by the Institutional Review Board of the sponsoring institution.

Fifty-three participants were enrolled in this study during an annual Sickle Cell Disease Research and Education Day event, and 9 participants were enrolled during clinic or day hospital visits at the sponsoring medical institution. Informed consent was obtained from all participants, including caregiver consent and patient assent. Participants age 18 and older also were consented. All participants were compensated for their time and effort.

Measures

Responsibility Scale for Pediatric Sickle Cell Disease-Revised. The Responsibility for Pediatric Sickle Cell Disease-Revised is a parent and youth-reported measure developed by the research team to assess level of involvement in and responsibility for daily disease management tasks specific to SCD. The current authors developed and revised the Responsibility Scale for Pediatric Sickle Cell Disease (Oliver-Carpenter, et al., 2011). The version used in this study included changes to the original version, including rewording and removing some of the items
and simplifying the Likert Scale to reduce response errors and missing data. The adapted version was more closely aligned to the validated and highly-researched Diabetes Family Responsibility Questionnaire (Anderson, et al., 1990) and Asthma Responsibility Questionnaire (McQuaid, et al., 2001).

The purpose of the revised measure was to assess caregiver and youth responsibility for tasks related to management of pediatric SCD during a typical day. Respondents were asked to rate the level of caregiver and youth responsibility on a 5-point scale. For 20 tasks, participants chose whether 1) *Parent takes responsibility all of the time*, 2) *Parent takes responsibility most of the time*, 3) *Parent and child share responsibility about equally*, 4) *Child takes responsibility most of the time*, or 5) *Child takes responsibility all of the time*. Alternatively, participants could report that nobody takes responsibility for the task, or that the task is not part of their treatment plan. This measure has one composite score, which is the mean of all 20 responses (ranging from 1 to 5). Higher scores indicate that youth are taking increased levels of responsibility. This measure has not yet been validated, although in this study, it demonstrated excellent internal consistency in caregiver (α = 0.93) and child reports (α = 0.93). Caregivers and youth completed this questionnaire separately. Of note, tasks identified as not part of participants’ treatment plans were coded as “Not Applicable”, and not included in the mean composite score. The inclusion of a “Not Applicable” response option was crucial because treatment plans for SCD tend to be highly individualized.

**McMaster Family Assessment Device General Functioning Subscale.** The McMaster Family Assessment Device (FAD; (Epstein, Baldwin, & Bishop, 1983) contains 60 questions examining family functioning. For this study, only the General Functioning Subscale was used, which contains 12 items evaluating general family functioning. Responses for the FAD ranged
from 1) **Strongly Agree** to 4) **Strongly Disagree**. Half of the items describe healthy family functioning and are reverse-scored to reduce acquiescence bias. Composite scores can range from 1.00 to 4.00, with scores below 2.00 indicating healthy family functioning. This measure has demonstrated adequate internal consistency and test-retest reliability and has been used in number of pediatric sickle cell studies (Barakat, et al., 2005; Barakat, et al., 2002; Lutz, et al., 2004; Mitchell, et al., 2007). Only caregivers completed this measure.

**Child Behavior Checklist (ages 8-18) & Youth Self Report Forms (ages 11-18).** The Child Behavior Checklist (CBCL; (Achenbach, 1991) is a 113-item scale evaluating internalizing and externalizing behavior, and social functioning. Youth ages 11 to 18 may fill out a self-report version of the measure called the Youth Self Report (YSR). For each item respondents rate whether the item occurs 0) **Never**, 1) **Sometimes**, or 2) **Frequently**. This measure has been extensively examined for reliability and validity, and it has been normed using both clinical and normative populations (Achenbach, 1991). This analysis utilized the empirically-derived Syndrome Scales (See Table 5). The eight Syndrome Scales and their composite Internalizing and Externalizing Scales, have demonstrated good internal consistency, ranging from alpha = .67 to .94. In this analysis, the Externalizing Composite Score (comprised of the Rule Breaking Behavior and Aggressive Behavior Syndrome Scales) was used in addition to the following Syndrome Scales: Anxious/Depressed, Withdrawn/Depressed, and Social Problems. Of note, we did not utilize the Internalizing Composite Score (comprised of the Anxious/Depressed, Withdrawn/Depressed, and Somatic Complaints Syndrome Scores) because the Somatic Complaints scores were inflated in individuals with more severe disease symptoms, but the intention of this measure was to examine psychosocial adjustment, not disease symptomology.
The CBCL has been used extensively in populations of children with chronic illnesses, such as HIV (Buchanas et al., 2001).

Some youth who participated in this study had ages outside the range of ages for which the YSR (ages 11-18) and CBCL (ages 8-18) are valid. Therefore, only a subset of participants was included in analyses involving the CBCL ($N = 51$) and YSR ($N = 34$).

**Family Information Form.** Caregivers completed a Family Information Form, which assessed demographic information and the patient’s medical status, including frequency of symptoms and school absences over the past 12 months. Additionally, caregivers were asked to rate the severity of their child’s SCD; possible responses were 1) *Mild*, 2) *Moderate*, and 3) *Severe*. Both caregivers and youth were asked how confident they were that the patient could manage their SCD on their own on a scale of 1) *Not Confident at All* to 5) *Very Confident*.

**Health care utilization.** Electronic medical records were reviewed to determine the number of ER visits and number of days spent inpatient due to complications of SCD in the 12 months before study enrollment. Only unplanned inpatient stays were included (e.g. planned inpatient stays after elective procedures were not included). The number of routine clinic visits recommended by the health care team for each participant in the year prior to enrollment was determined using electronic medical records as well. Medical record data was not available for two of the participants because they had transferred to the adult sickle cell clinic at a separate institution, which utilizes a different electronic medical record system.

**Data Management**

The current analyses included data from 61 participants with complete and usable data, although one participant was excluded due to extreme values (disease trajectory and severity measures more than three standard deviations from the mean). To be used in the analyses,
responsibility measures needed to be complete, although participants above the age of 18 were not required to have participating caregivers. The CBCL and YSR measures were completed by a subset of participants from the overall sample (87.9% and 55.7% respectively). Participants who completed the YSR measure were representative of the larger sample on age, gender, and hemoglobin type; however, caregivers who completed the CBCL had participant youth who were representative on gender and hemoglobin type, but were significantly younger than participants who did not complete the measure. This was because caregivers generally did not complete the CBCL because their children were older than 18, and the CBCL has not been normed and is generally not used above this age.

**Statistical Analyses**

All statistical analyses were conducted using PASW for Windows version 18.0. In the analyses, responses that were left blank or included more than one response were coded as “missing data”. Descriptive statistics were generated for demographics, type of SCD, healthcare utilization, recommended number of clinic visits, days with SCD pain, and school absences. Composite Responsibility Scores were generated for caregiver and youth reports; descriptive analyses for each item of the measure were generated to understand involvement in detail, as there is little SCD literature dealing with responsibility for all of the tasks in the measure. In addition, descriptive statistics were also generated for family functioning and child psychosocial well-being. Pearson correlations were utilized to examine the relationship between caregiver and youth responsibility for disease management and youth age, youth psychosocial functioning, family functioning, and urgent healthcare utilization. Spearman correlations were utilized for analyses involving hemoglobin type because hemoglobin type was coded as a binary variable (whether or not participants had the most severe hemoglobin type). Post-hoc multiple regression
analyses were used to examine the utility of age, psychosocial, and health variables in predicting the level of caregiver and youth responsibility for disease management.

Responsibility Scale Composite Score and responsibility items were coded as continuous variables, consistent with the coding systems of validation and seminal studies using the DFRQ and ARQ (Anderson, et al., 1990; Helgeson, et al., 2008; Holmes, et al., 2006; McQuaid, et al., 2001; Palmer, et al., 2009). Age was used as a continuous variable in correlational and regression analyses; however, in order to gain a better understanding of responsibility for disease management in different age groups, age was used as a categorical variable in the independent sample t-tests comparing youth report of responsibility of each task in children (ages 8-12 years) and young adults (ages 13-21 years).
CHAPTER 3

Results

Preliminary Analyses

Visual inspection of histograms and stem-and-leaf plots were used to assess for violations of assumptions. Several variables regarding health care utilization and disease symptoms did not meet normality assumptions, and several outliers were noted. Extreme outliers were removed; however, non-normality was not addressed, as it only occurred to a high degree in ER visits, and this is expected, due to the nature of variability in illness presentation in SCD.

Participant Demographics

The current analysis includes 61 youth with SCD and 58 caregivers. Three young-adults with SCD, 18 years and above participated in the study without caregiver involvement. The youth participants were a mean of 13.13 years old ($SD = 3.63$), and 52.5% were female. Sixty-seven percent of caregivers reported being single or separated/divorced and 63.3% reported being the only adult caregiver in their household (See Table 1). Eighty-three percent of the caregivers were mothers, 8.6% were fathers, and 8.5% were other. Other caregivers included grandmothers ($N = 2$), aunt ($N = 1$), stepfather ($N = 1$), and other ($N = 1$). The majority of caregivers (95%) reported being African American/Black, and all youth participants reported being African American/Black. The majority of households in this sample reported a total annual income of under $50,000 (82.46%) and most caregivers did not have a college or trade school degree (71.43%).

Descriptive Results

Disease severity. Seventy-five percent of youth participants had Hemoglobin SS Disease (HgSS), 18.0% had Hemoglobin SC (HgSC), and the remaining 6.6% had Hemoglobin Sickle
Beta+Thalassemia (See Table 2). Of note, HgSS tends to be the genotype with the most severe trajectory, although this may vary and genotype is not the single determining factor in severity (Gil et al., 2001). Thirty-two percent of caregivers reported that their child’s SCD course was mild, 38.33% moderate, and 28.33% severe. Of note, one participant was experiencing an extremely severe disease trajectory, and therefore was excluded from the study due to extreme values that skewed some of the results. This participant had a total of 66 inpatient days and nine ER visits. Additionally, the sickle cell care team recommended that this patient come to clinic 19 times in the year before study enrollment; at some points, it was requested that she come in three times per week.

**Health care utilization.** Table 2 outlines mean number of ER visits, inpatient days, and clinic visits in the previous year. Of note, the SCD care team recommended that 30.6% of participants visit day hospital or clinic every three to four weeks. This type of treatment requires a great deal of commitment, as day hospital visits (usually for blood transfusions or apheresis) can take up to seven hours.

**Disease management responsibility patterns.** As predicted, caregiver and youth reports of disease management responsibility were moderately correlated ($r = 0.57, p = .00$), and caregivers tended to perceive that their children took less responsibility than the children themselves reported taking. On a five-point scale from 1 (*Parent takes responsibility all of the time*) to 5 (*Child takes responsibility all of the time*), composite responsibility scores ranged from 1.00 to 4.05 for caregiver report and 1.00 to 5.00 for youth report, with a mean of 2.45 ($SD = 0.90$) for child report, and 2.13 ($SD = 0.80$) for caregiver report. This indicates that, in general, caregivers tended to take most of the responsibility for disease management, as low scores indicate that the caregiver takes the most responsibility, while high scores indicate that the youth
has the most responsibility. A score of approximately three indicates that responsibility is shared equally.

Caregivers tended to report taking the most responsibility for making appointments with dentists and other doctors, remembering clinic appointments, explaining school absences, and telling teachers about SCD. Youth reported sharing responsibility with their caregivers for tasks such as drinking sufficient fluids and making decisions about adjustments in activity when symptoms occur. Mean responsibility scores for each task are presented in Table 3.

**Family functioning.** The FAD General Functioning scores ranged from 1.58 to 2.58, with a mean of 1.57 (SD = 0.40). Most of the families had scores in the “healthy” range (82.76%), indicating that the participants in this sample generally had good family functioning, similar to normative populations (Kabacoff, Miller, Bishop, Epstein, & Keitner, 1990).

**Child psychosocial functioning.** Participant youth tended to have good psychosocial functioning, as measured by the CBCL and YSR Syndrome Scales and Internalizing and Externalizing Scales (See Table 4). As expected, participants had higher Somatic Complaint Scores than non-chronically ill populations, as demonstrated by YSR and CBCL norms, which resulted in inflated Internalizing Behavior Scores. Excluding the Somatic Complaint and Internalizing Behavior Scales, the percentage of participants who had T Scores above 65 for the remaining Syndrome Scales ranged from 4% (Aggressive Behavior) to 10% (Anxious/Depressed) for the CBCL and 6.1% (Rule Breaking Behaviors) to 14.7% (Withdrawn/Depressed) for the YSR.

**Aim 1: Responsibility and Age**

As predicted, increased age was moderately correlated with caregiver ($r = 0.52, p = .00$) and youth report ($r = 0.64, p = .00$) of greater youth responsibility for disease management.
Independent sample T-Tests revealed that teenagers and young adults (ages 13-21 years) reported significantly higher levels of responsibility for disease management compared to children (ages 8-12 years) on 15 out of the 20 tasks on the Responsibility Scale (See Table 5). The composite score for teens and young adults indicates that responsibility for disease management was generally shared ($M = 2.89$, $SD = 0.97$), but that caregivers still appear to have slightly more responsibility than their teenage and young adult children. Teenagers and young adults reported sharing responsibility with their caregiver for seven of the tasks in the Responsibility Scale (See Figure 2; A score of three indicates sharing). It is important to note, though, that caregivers continue to take an active role in disease management even as their children get older. Table 4 provides a comparison of mean responsibility scores for each disease management task between children (ages 8-12 years) and teenagers and young adults (ages 13-21 years).

**Aim 2: Responsibility and Youth and Family Psychosocial Functioning**

Youth psychosocial functioning, as measured by the CBCL, was associated with disease management responsibility in several ways. First, youth self-report of higher level of Externalizing Behaviors was associated with caregiver report that the youth was taking less responsibility for their own illness management ($r = 0.36$, $p = .04$). Additionally, caregiver report of increased Withdrawn/Depressed symptoms was associated with child report of less responsibility for their own disease management ($r = 0.28$, $p = .05$). These findings support our hypothesis that children with worse psychosocial functioning would have less responsibility for disease self-care, but the pattern of associations is not consistent across youth and caregiver reports. Disease management responsibility was not associated with family functioning. These findings are summarized in Table 6.
Aim 3: Responsibility and Hemoglobin Type and Health Care Utilization

Although we hypothesized that worse disease severity, as measured by genotype, would be associated with greater caregiver responsibility for disease management, neither caregiver nor youth report of responsibility was associated with hemoglobin type in a Spearman correlation ($p > .05$; See Table 7). Of note, increased levels of self-reported responsibility by youth was associated with higher numbers of ER visits ($r = 0.34, p = .01$), suggesting some sort of association with disease symptomology. This association, though, was not consistent. For example, caregiver report of responsibility was not associated with ER visits ($r = 0.10, p = .45$). These findings are summarized in Table 7.

Post-Hoc Analyses

Hierarchical linear regressions were run individually for youth and caregiver report to determine whether age, psychosocial, and health variables had utility in predicting level of responsibility for disease management tasks. For the prediction of youth report of responsibility, a model including age, CBCL Withdrawn/Depressed Score, and ER visits, was determined to be the best model as it predicted 30% of the variance $F(49) = 6.48, p = .00$ (See Table 8). For caregiver report of responsibility, a model including only age and YSR Externalizing Score was determined as optimal, as it predicted 20% of the variance in caregiver report of responsibility $F(33) = 4.70, p = .02$ (See Table 9).
Table 1.

**Summary of Categorical Participant Demographics**

<table>
<thead>
<tr>
<th>Patient Characteristics</th>
<th>Percentage</th>
<th>N</th>
</tr>
</thead>
<tbody>
<tr>
<td><strong>Child Gender (N = 61)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Female</td>
<td>52.50%</td>
<td>32</td>
</tr>
<tr>
<td>Male</td>
<td>47.50%</td>
<td>29</td>
</tr>
<tr>
<td><strong>Type of Sickle Cell Disease (N = 61)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>HgSS</td>
<td>75.40%</td>
<td>46</td>
</tr>
<tr>
<td>HgSC</td>
<td>18.00%</td>
<td>11</td>
</tr>
<tr>
<td>HgSB+Thal</td>
<td>6.60%</td>
<td>4</td>
</tr>
<tr>
<td><strong>Caregiver Marital Status (N = 57)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Single</td>
<td>56.14%</td>
<td>32</td>
</tr>
<tr>
<td>Married</td>
<td>31.58%</td>
<td>18</td>
</tr>
<tr>
<td>Separated/Divorced</td>
<td>10.53%</td>
<td>6</td>
</tr>
<tr>
<td>Widowed</td>
<td>1.70%</td>
<td>1</td>
</tr>
<tr>
<td><strong>Caregiver Relation to Participant (N = 58)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Mother</td>
<td>82.80%</td>
<td>48</td>
</tr>
<tr>
<td>Father</td>
<td>8.60%</td>
<td>5</td>
</tr>
<tr>
<td>Grandmother</td>
<td>3.40%</td>
<td>2</td>
</tr>
<tr>
<td>Aunt</td>
<td>1.70%</td>
<td>1</td>
</tr>
<tr>
<td>Stepfather</td>
<td>1.70%</td>
<td>1</td>
</tr>
<tr>
<td>Other</td>
<td>1.70%</td>
<td>1</td>
</tr>
<tr>
<td><strong>Caregiver Education (N = 56)</strong></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Less than High School</td>
<td>16.07%</td>
<td>9</td>
</tr>
<tr>
<td>High School Graduate</td>
<td>19.60%</td>
<td>11</td>
</tr>
<tr>
<td>Partial College</td>
<td>35.70%</td>
<td>20</td>
</tr>
<tr>
<td>Trade School</td>
<td>3.60%</td>
<td>2</td>
</tr>
<tr>
<td>College Graduate</td>
<td>25.00%</td>
<td>14</td>
</tr>
</tbody>
</table>
Table 2.

*Summary of Patient Demographics (N = 61)*

<table>
<thead>
<tr>
<th>Patient Characteristics</th>
<th>Mean</th>
<th>(SD)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Age</td>
<td>13.13</td>
<td>(3.65)</td>
</tr>
<tr>
<td>Recommended number of clinic visits (in the past 12 months)</td>
<td>6.66</td>
<td>(4.79)</td>
</tr>
<tr>
<td>Adherence to recommended clinic visits (in percentage)</td>
<td>81.53</td>
<td>(26.84)</td>
</tr>
<tr>
<td>ER Visits (in the past 12 months)</td>
<td>1.66</td>
<td>(1.64)</td>
</tr>
<tr>
<td>Inpatient days (in the past 12 months)</td>
<td>3.42</td>
<td>(4.92)</td>
</tr>
<tr>
<td>Number of days with SCD Pain (in the last 12 months)</td>
<td>30.16</td>
<td>(60.97)</td>
</tr>
<tr>
<td>Number of pain crises (in the last 12 months)</td>
<td>6.30</td>
<td>(10.26)</td>
</tr>
<tr>
<td>School absences (in last school year)</td>
<td>12.15</td>
<td>(14.57)</td>
</tr>
</tbody>
</table>
Table 3.
Responsibility for Disease Management Tasks: Comparison of Parent and Child Reports ($N = 61$)

<table>
<thead>
<tr>
<th>Task</th>
<th>Parent Report $M$ ($SD$)</th>
<th>Child Report $M$ ($SD$)</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. Noticing early signs of an acute crisis</td>
<td>2.36 (1.18)</td>
<td>2.62 (1.46)</td>
</tr>
<tr>
<td>2. Starting treatment when symptoms occur</td>
<td>2.07 (1.11)</td>
<td>2.17 (1.25)</td>
</tr>
<tr>
<td>3. Taking daily preventative medication as directed by doctor</td>
<td>2.47 (1.37)</td>
<td>2.39 (1.47)</td>
</tr>
<tr>
<td>4. Noticing when medications are beginning to run out and will need</td>
<td>1.96 (1.27)</td>
<td>2.20 (1.38)</td>
</tr>
<tr>
<td>to be refilled soon</td>
<td></td>
<td></td>
</tr>
<tr>
<td>5. Making sure child stays warm in certain situations</td>
<td>2.16 (1.04)</td>
<td>2.87 (1.44)</td>
</tr>
<tr>
<td>6. Avoiding situations that may make symptoms more likely</td>
<td>2.09 (1.11)</td>
<td>2.50 (1.36)</td>
</tr>
<tr>
<td>7. Remembering to take medicine along if child will be away from</td>
<td>2.18 (1.35)</td>
<td>2.68 (1.55)</td>
</tr>
<tr>
<td>home in case of symptom onset</td>
<td></td>
<td></td>
</tr>
<tr>
<td>8. Telling teachers about the child’s sickle cell disease</td>
<td>1.89 (1.17)</td>
<td>2.17 (1.39)</td>
</tr>
<tr>
<td>9. Making decisions about adjustments in activity when symptoms</td>
<td>2.39 (1.18)</td>
<td>2.88 (1.52)</td>
</tr>
<tr>
<td>occur</td>
<td></td>
<td></td>
</tr>
<tr>
<td>10. Drinking sufficient fluids throughout the day</td>
<td>3.16 (1.54)</td>
<td>3.34 (1.48)</td>
</tr>
<tr>
<td>11. Remembering day of clinic appointment</td>
<td>1.64 (0.97)</td>
<td>1.93 (1.33)</td>
</tr>
<tr>
<td>12. Making appointments with dentists and other doctors</td>
<td>1.34 (0.85)</td>
<td>1.64 (1.20)</td>
</tr>
<tr>
<td>13. Telling relatives about sickle cell disease</td>
<td>2.02 (0.97)</td>
<td>2.59 (1.35)</td>
</tr>
<tr>
<td>14. Noticing differences in health, such as weight changes or signs</td>
<td>2.11 (1.06)</td>
<td>2.32 (1.42)</td>
</tr>
<tr>
<td>of an infection</td>
<td></td>
<td></td>
</tr>
<tr>
<td>15. Telling friends about sickle cell disease</td>
<td>2.34 (1.34)</td>
<td>3.24 (1.60)</td>
</tr>
<tr>
<td>16. Explaining absences from school to teachers or other school</td>
<td>1.71 (1.24)</td>
<td>2.14 (1.29)</td>
</tr>
<tr>
<td>personnel</td>
<td></td>
<td></td>
</tr>
<tr>
<td>17. Remembering to take ex-jade or other chelation medication</td>
<td>2.16 (1.28)</td>
<td>2.14 (1.34)</td>
</tr>
<tr>
<td>18. Taking vitamins and minerals daily</td>
<td>2.17 (1.43)</td>
<td>2.69 (1.49)</td>
</tr>
<tr>
<td>19. Taking more or less pain medication according to pain levels</td>
<td>1.96 (1.20)</td>
<td>2.09 (1.30)</td>
</tr>
<tr>
<td>and other symptoms</td>
<td></td>
<td></td>
</tr>
<tr>
<td>20. Finding other behavioral strategies to manage pain</td>
<td>2.18 (1.22)</td>
<td>2.53 (1.33)</td>
</tr>
</tbody>
</table>

Bold items indicate the items for which caregivers reported taking the most responsibility. Italicized items indicate items for which youth reported shared responsibility.
Table 4.

*Frequency of Participants with T-Score of 65 and above for Syndrome Scales*

<table>
<thead>
<tr>
<th>Syndrome</th>
<th>CBCL N = 50 Percentage (N)</th>
<th>YSR N = 34 Percentage (N)</th>
</tr>
</thead>
<tbody>
<tr>
<td>Anxious/Depressed</td>
<td>10% (N = 5)</td>
<td>8.8% (N = 3)</td>
</tr>
<tr>
<td>Withdrawn/Depressed</td>
<td>7.9% (N = 4)</td>
<td>14.7% (N = 5)</td>
</tr>
<tr>
<td>Somatic Complaints</td>
<td>41.4% (N = 21)</td>
<td>22.9% (N = 8)</td>
</tr>
<tr>
<td>Internalizing Behaviors</td>
<td>21.7% (N = 11)</td>
<td>14.5% (N = 5)</td>
</tr>
<tr>
<td>Social Problems</td>
<td>8.0% (N = 4)</td>
<td>8.7% (N = 3)</td>
</tr>
<tr>
<td>Rule Breaking</td>
<td>5.9% (N = 3)</td>
<td>6.1% (N = 2)</td>
</tr>
<tr>
<td>Aggressive Behavior</td>
<td>4.0% (N = 2)</td>
<td>8.7% (N = 3)</td>
</tr>
<tr>
<td>Externalizing Behaviors</td>
<td>4.0% (N = 2)</td>
<td>8.7% (N = 3)</td>
</tr>
</tbody>
</table>
Table 5.
Responsibility for Disease Management Tasks: Independent Sample T-Tests Comparing Child and Young Adult Self-Report

<table>
<thead>
<tr>
<th>Task Description</th>
<th>Ages 8-12 M (SD)</th>
<th>Ages 13-21 M (SD)</th>
<th>t</th>
<th>p</th>
</tr>
</thead>
<tbody>
<tr>
<td>1. Noticing early signs of an acute crisis</td>
<td>2.27 (1.29)</td>
<td>2.97 (1.56)</td>
<td>.06</td>
<td>-1.89</td>
</tr>
<tr>
<td>2. Starting treatment when symptoms occur</td>
<td>1.62 (0.98)</td>
<td>2.70 (1.26)</td>
<td>.00</td>
<td>-3.66</td>
</tr>
<tr>
<td>3. Taking daily preventative medication as directed by doctor</td>
<td>1.79 (1.08)</td>
<td>2.97 (1.59)</td>
<td>.00</td>
<td>-3.33</td>
</tr>
<tr>
<td>4. Noticing when medications are beginning to run out and will need to be refilled soon</td>
<td>1.43 (0.77)</td>
<td>2.97 (1.43)</td>
<td>.00</td>
<td>-5.18</td>
</tr>
<tr>
<td>5. Making sure child stays warm in certain situations</td>
<td>2.23 (1.31)</td>
<td>3.13 (1.46)</td>
<td>.01</td>
<td>-2.52</td>
</tr>
<tr>
<td>6. Avoiding situations that may make symptoms more likely</td>
<td>2.23 (1.25)</td>
<td>2.77 (1.43)</td>
<td>.13</td>
<td>-1.54</td>
</tr>
<tr>
<td>7. Remembering to take medicine along if child will be away from home in case of symptom onset</td>
<td>2.25 (1.43)</td>
<td>3.08 (1.58)</td>
<td>.04</td>
<td>-2.10</td>
</tr>
<tr>
<td>8. Telling teachers about the child’s sickle cell disease</td>
<td>1.66 (1.01)</td>
<td>2.67 (1.54)</td>
<td>.04</td>
<td>-3.00</td>
</tr>
<tr>
<td>9. Making decisions about adjustments in activity when symptoms occur</td>
<td>2.55 (1.45)</td>
<td>3.20 (1.54)</td>
<td>.10</td>
<td>-1.66</td>
</tr>
<tr>
<td>10. Drinking sufficient fluids throughout the day</td>
<td>3.07 (1.51)</td>
<td>3.60 (1.43)</td>
<td>.16</td>
<td>-1.41</td>
</tr>
<tr>
<td>11. Remembering day of clinic appointment</td>
<td>1.63 (1.07)</td>
<td>2.23 (1.50)</td>
<td>.08</td>
<td>-1.78</td>
</tr>
<tr>
<td>12. Making appointments with dentists and other doctors</td>
<td>1.27 (0.83)</td>
<td>2.00 (1.39)</td>
<td>.02</td>
<td>-2.51</td>
</tr>
<tr>
<td>13. Telling relatives about sickle cell disease</td>
<td>2.07 (1.18)</td>
<td>3.07 (1.34)</td>
<td>.00</td>
<td>-3.00</td>
</tr>
<tr>
<td>14. Noticing differences in health, such as weight changes or signs of an infection</td>
<td>1.80 (1.06)</td>
<td>2.86 (1.53)</td>
<td>.00</td>
<td>-3.11</td>
</tr>
<tr>
<td>15. Telling friends about sickle cell disease</td>
<td>2.82 (1.64)</td>
<td>3.71 (1.43)</td>
<td>.05</td>
<td>-2.06</td>
</tr>
<tr>
<td>16. Explaining absences from school to teachers or other school personnel</td>
<td>1.57 (0.82)</td>
<td>2.87 (1.36)</td>
<td>.00</td>
<td>-4.49</td>
</tr>
<tr>
<td>17. Remembering to take ex-jade or other chelation medication</td>
<td>1.61 (1.08)</td>
<td>2.71 (1.38)</td>
<td>.01</td>
<td>-2.97</td>
</tr>
<tr>
<td>18. Taking vitamins and minerals daily</td>
<td>2.24 (1.36)</td>
<td>3.17 (1.50)</td>
<td>.03</td>
<td>-2.27</td>
</tr>
<tr>
<td>19. Taking more or less pain medication according to pain levels and other symptoms</td>
<td>1.62 (0.94)</td>
<td>2.55 (1.45)</td>
<td>.01</td>
<td>-2.99</td>
</tr>
<tr>
<td>20. Finding other behavioral strategies to manage pain</td>
<td>2.45 (1.33)</td>
<td>2.60 (1.35)</td>
<td>.67</td>
<td>-0.45</td>
</tr>
</tbody>
</table>
Figure 2. Comparison of Responsibility by Age Group. Self-report of responsibility for the teen and young adult group (ages 13-21) and the child group (ages 8-12) are shown above. The teen and young adult group reported sharing of 7 tasks above, as indicated by bars that approach or pass the red line, which indicates shared responsibility.
### Table 6.

**Correlation of Psychosocial and Family Functioning and Responsibility**

<table>
<thead>
<tr>
<th></th>
<th>Caregiver Report</th>
<th>Youth Report</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td><em>r</em></td>
<td><em>p</em></td>
</tr>
<tr>
<td>Externalizing Youth Report</td>
<td>-0.36</td>
<td>.04</td>
</tr>
<tr>
<td>Withdrawn/Depressed Caregiver Report</td>
<td>0.08</td>
<td>.58</td>
</tr>
<tr>
<td>Family Functioning</td>
<td>0.05</td>
<td>.69</td>
</tr>
</tbody>
</table>

### Table 7.

**Correlation of Health Correlates and Responsibility**

<table>
<thead>
<tr>
<th></th>
<th>Caregiver Report</th>
<th>Youth Report</th>
</tr>
</thead>
<tbody>
<tr>
<td></td>
<td><em>r</em></td>
<td><em>p</em></td>
</tr>
<tr>
<td>Hemoglobin Type</td>
<td>0.20</td>
<td>.13</td>
</tr>
<tr>
<td>ER Visits</td>
<td>0.10</td>
<td>.49</td>
</tr>
<tr>
<td>Hospitalizations</td>
<td>0.10</td>
<td>.45</td>
</tr>
</tbody>
</table>
Table 8.

Hierarchical Linear Regression Models Predicting Youth Report of Responsibility

<table>
<thead>
<tr>
<th></th>
<th>B</th>
<th>SEB</th>
<th>β</th>
<th>R²</th>
<th>Δ R²</th>
<th>F</th>
</tr>
</thead>
<tbody>
<tr>
<td>Step 1: Age</td>
<td>0.12</td>
<td>0.03</td>
<td>0.17</td>
<td>.21</td>
<td>.21</td>
<td>12.56</td>
</tr>
<tr>
<td>Step 2: Age</td>
<td>0.11</td>
<td>0.03</td>
<td>0.15</td>
<td>.24</td>
<td>.04</td>
<td>7.51</td>
</tr>
<tr>
<td></td>
<td>-0.03</td>
<td>0.02</td>
<td>0.23</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td>Step 3: Age</td>
<td>-0.11</td>
<td>0.03</td>
<td>-0.10</td>
<td>.30</td>
<td>.06</td>
<td>6.48</td>
</tr>
<tr>
<td></td>
<td>-0.03</td>
<td>0.02</td>
<td>0.22</td>
<td></td>
<td></td>
<td></td>
</tr>
<tr>
<td></td>
<td>0.11</td>
<td>0.06</td>
<td>0.39</td>
<td></td>
<td></td>
<td></td>
</tr>
</tbody>
</table>

Table 9.

Hierarchical Linear Regression Models Predicting Caregiver Report of Responsibility

<table>
<thead>
<tr>
<th></th>
<th>B</th>
<th>SEB</th>
<th>β</th>
<th>R²</th>
<th>Δ R²</th>
<th>F</th>
</tr>
</thead>
<tbody>
<tr>
<td>Step 1: Age</td>
<td>0.09</td>
<td>0.05</td>
<td>0.30</td>
<td>.09</td>
<td>.09</td>
<td>3.19</td>
</tr>
<tr>
<td>Step 2: Age</td>
<td>0.10</td>
<td>0.05</td>
<td>0.32</td>
<td>.23</td>
<td>.14</td>
<td>4.70</td>
</tr>
<tr>
<td></td>
<td>-0.02</td>
<td>0.01</td>
<td>-0.38</td>
<td></td>
<td></td>
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</tr>
</tbody>
</table>
CHAPTER 4

Discussion

Managing a chronic illness such as SCD requires effective coordination of disease management tasks within families. The current study examined how youth and caregivers manage disease-related tasks in SCD and how their level of responsibility for these tasks may be related to age, youth and family psychosocial functioning, and illness severity (measured by hemoglobin type and urgent health care utilization).

As expected, caregivers tended to have more responsibility for disease management than youth, especially for tasks involving navigation of the health care and school systems, such as making appointments with dentists and other doctors, remembering day of clinic appointment, and explaining absences from school to teachers or other school personnel. Youth reported sharing responsibility for tasks having to do with symptom self-monitoring and decision-making, including drinking sufficient fluids, making decisions about adjusting activity when symptoms occur, and staying warm. Caregivers tended to rate youths’ responsibility for tasks lower than did youth; however, youth and caregivers generally agreed on the level of responsibility for tasks for which caregivers took high levels of responsibility. This finding is likely developmentally appropriate, but may result in differences in perception, as youth may feel that their caregivers may not appreciate how much they are monitoring symptoms and making disease-related decisions on a day-to-day basis. On the other hand, caregivers may be doing more than youth realize in managing daily illness management tasks.

Older youth tended to report having more responsibility than younger youth, although caregivers still retained a substantial amount of responsibility, even into early adulthood. Older youth generally reported a pattern of shared responsibility for a great deal of tasks. The findings
from this study are consistent with the pediatric literature, which suggests that parents remain involved in disease management as youth get older, although their involvement may change from actually helping the child engage in the task, to periodic supervision of an older child. In this sample, caregivers remained active in disease management longer than indicated in several other studies, where youth with other chronic illnesses have been cited to take primary responsibility between the ages of 15 to 17 (Orrell-Valente, et al., 2008; Schilling, et al., 2006). In comparison, in this study, caregivers continued to take primary responsibility, even in older adolescents. Consistent with the current findings, youth with HIV may also take responsibility for disease management at an older age, as one study with a predominantly minority sample (86%) found that caregivers took primary responsibility for youth medication administration for most participants (Naar-King, et al., 2009). Further research will be needed to examine the role of cultural and economic factors, as the literature suggests that African American parents tend to differ from White parents on degree of autonomy granting and parental control (Smetana, 2011).

The data did not support the hypothesis that family functioning would be associated with youth and caregiver level of responsibility. This was unexpected, given that the literature emphasizes that family collaboration, communication, and stability are important for effective disease management. Family members need to be able to work as a team (Wiebe, et al., 2005) to solve problems and integrate disease management into their daily routine (Fiese & Wamboldt, 2003). It is possible that there was no relationship between family functioning and responsibility in the current study due to lack of measure sensitivity. The majority of participants in the study had “healthy” family functioning scores, so a measure with more sensitivity in measuring variances of family functioning in the healthy range may have been more appropriate in this sample. Social desirability factors may also have affected the findings in the current study. One
final possibility is that the association of family factors with responsibility may require a more complex study design to better understand family functioning and responsibility for disease management and other factors involved.

It was also interesting that youth who reported having more responsibility tended to have experienced more ER visits in the previous year. However, caregiver report of responsibility was not associated with ER visits, suggesting that this association was specifically driven by youth perception of responsibility. One possible explanation of this finding is that youth with frequent urgent health care contacts tended to have a more severe disease trajectory and therefore perceived themselves as engaging in higher levels of disease management.

The hypothesis that youth with psychological problems would have less responsibility was partially supported in the current study, as externalizing behavior was associated with less youth responsibility. One possible interpretation is that youth with high levels of externalizing behaviors may not be perceived as competent or may not have the competencies required for effective disease management. This interpretation is supported by the literature suggesting that externalizing behavior is associated with worse adherence (Bryden, et al., 2001; Duke, et al., 2008; Holmes, et al., 2006; Horton, et al., 2009).

Family context may moderate the association between psychosocial adjustment and responsibility for disease management. In a healthy family context, caregivers may have the resources to compensate for a child with psychosocial problems, who is unable to properly manage their illness, whereas caregivers in an unhealthy family context may not have the resources to do so. Further research will be necessary to better elucidate this relationship and its short- and long-term implications for both psychosocial functioning and disease management and coping, especially within the context of family functioning. Additional research will be
necessary to further examine how the family context affects the association between youth age, psychosocial functioning, and responsibility for disease management.

The findings of this study are significant given the challenges for caregivers to balance youth desire and need for autonomy with the need to protect their child’s health and ensure oversight. The study findings are consistent with the existing literature and indicate the importance of continued youth-caregiver partnership, as well as the importance of communicating effectively with health providers.

**Study Limitations and Strengths**

Study limitations should be acknowledged. A limitation of the current study is the relatively small convenience sample. The sample size, though comparable to other published studies in SCD, limits power and effect size in the study, especially given the wide age range. A larger study is needed to validate and clarify the findings. Only a subset of the participants filled out the CBCL and YSR in the current study, so future studies that include measures of psychosocial adjustment for all participants will be necessary to better generalize the findings. The study’s strengths include that it presents pilot data from a revised scale examining roles and responsibility in management tasks specific to pediatric SCD. This measure was coded in a manner consistent with that of current validated responsibility measures in asthma and diabetes (coding responsibility as a continuous measure), but future validation studies may also consider the merits of an alternative categorical coding system (such as coding responsibility as low vs. high) as has been used in some studies utilizing non-validated measures (e.g. Naar-King et al., 2009). In light of the limitations, the study also expands the SCD literature and provides additional insights for clinical practice and future research. Future directions will contribute to an
understanding across broader developmental, family systems, cultural, and collaborative care frameworks.

**Future Directions and Implications**

**Implications for clinical practice.** Health care providers are in a unique position to assist in the process of shifting responsibility to youth by helping both caregivers and youth understand how to accomplish these tasks and suggesting ways in which caregivers can begin to shift responsibility for these tasks to youth during adolescence. Study findings highlight the need to coordinate care with families, as well as with providers to ensure optimal health outcomes. Discussions about the distribution of disease management responsibility within families should be an important component of routine clinic visits, as there is evidence that youth and caregivers do not generally communicate with each other about roles in disease management at home (Pai, et al., 2011). Interventions are needed to ensure that caregivers and youth are optimally involved in disease management and that tasks are transferred to youth over time, while also promoting adherence with the treatment regimen.

**Implications for research.** Future research with a larger sample size should examine involvement/disease management responsibility trajectories over the course of childhood, adolescence and into adulthood. As these studies emerge, we can better understand the age at which youth begin to manage illness-related tasks independently in SCD and how this compares to other chronic illnesses. Additionally, longitudinal methodology would allow for a better understanding of factors contributing to disease management and identification of the types of caregiver-youth management partnerships associated with the best outcomes. Another factor that would help in identifying patterns of disease management responsibility associated with the best outcomes would be adherence to recommended medical regimen. Future research involving
responsibility for disease management in SCD should include a measure of adherence in order to better elucidate the mechanisms connecting responsibility for disease management and health outcomes. Additionally, disease-specific research addressing family functioning will play a crucial role in increasing our understanding of disease management in the family context for youth with SCD.
References


management of pediatric medical treatment regimens. *Pediatric Transplantation, 14*(8), 993-999. doi: 10.1111/j.1399-3046.2010.01391.x


