IRB Protocol
CRC Course 2012
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A. Study Purpose and Rationale

Sickle Cell Anemia is a disease that affects between 80-90,000 African American and Hispanic patients in the U.S. each year. Of those about 40% are pediatric patients[1]. The morbidly from Sickle Cell Anemia is caused by pain crisis's and organ damage resulting in multiple hospitalizations and a decreased quality of life. Hydroxyurea (HU) is the only FDA approved medication for treating Sickle Cell Anemia which has been shown to decrease the morbidity, number of hospital admissions and to improve quality of life[2]. Despite the considerable health benefits of HU to Sickle Cell patients, adherence remains poor and therefore the complete effects of the medication are not necessarily achieved in patients who are prescribed the drug[3]. When used as prescribed the health benefits of HU are reduction in symptoms and improvement in quality of life[4]. These usually do not become apparent until 3-6 months after the start of therapy. Markers of HU adherence that can be used are the highest level of Fetal Hemoglobin (HgbF) achieved by an individual patient. When assessed regularly adherence can be determined by maintenance of HgbF level. Other markers that are used to assess adherence are pharmacy refill data and patient self-reported adherence[3].

A preliminary data gathering survey was conducted among the patients of the Columbia Sickle Cell clinic. A convenience sample of 42 patients who were on HU was selected to assess adherence based on HgbF levels. It was shown that only 31% of the patients sampled were within 10% of their target HgbF. These patients were all on HU for 2 or more years and HbF was assessed on average 5 times a year.

A survey assessing parental barriers to HU adherence was performed on 173 parents. The survey showed that the majority (63%) of respondents had never heard of HU and even less knew that HU reduces pain crises. Concerns regarding drug efficacy and safety were identified as major barriers to medication adherence. This preliminary data suggests that parents are more aware of the potential adverse effects of HU and not nearly as informed about the benefits of the drug which could potentially be a barrier to HU regimen compliance.

Medication non adherence with HU in the Sickle Cell population is well known and in an effort to improve adherence there is an intervention study that will be implemented at Columbia University after funding approval using a combination of Community Health Workers and text messaging to try and address the barriers to HU adherence. This risk assessment study will be a precursor to the intervention to establish barriers to medication use and to obtain patient attitudes regarding texting utilization and suggestions on personal methods of improving adherence that have the possibility of being used to create other interventions to improve adherence.

The purpose of this study is to gain a better understanding of risk factors for poor medication adherence among HU patients and their parents so that patients can receive the maximum benefit of the medication. Another aim is to assess patient attitudes/comfort with text messaging as a system to improve HU adherence and to gain information on what content they would prefer in the messages. Lastly this study aims to assess if any patients or parents have any innovative ideas for improving HU adherence that could be used on a large scale.

B. Study Design and statistical Analysis

This study will be a Cross sectional design. Subjects will be selected from the Columbia Sickle Cell Clinic roster. There are 191 patients that receive care in Columbia's Pediatric Sickle Cell Clinic. Of those about 59 are on HU therapy and are between the ages of 10-17 and about 40 would meet inclusion criteria.

Patients who are also on Exjade will be included in the study since the population is similar and both groups take daily medication whose effects are related to long term adherence. Patients and parents must be able to speak English or Spanish. Consent will be obtained at clinic visits. Patients and parents will undergo phone surveys for risk assessment. The surveys will assess several potential risk factors including age, sex, transportation costs, parental education, parental socioeconomic status, family support, appointment no show rate, medication self-management, forgetfulness, developmental delay, fear of adverse effects, lack of knowledge of benefits and sibling with SCD or other chronic disease.

Data will be collected from pharmacies to obtain medication refill data and a chart review will be conducted to obtain HgbF data for those patients on HU. An unpaired t-test will be used to determine if any of the assessed risk factors correlate with medication adherence. A power analysis shows that by using the 42 patients on HU that meet inclusion criteria and the estimated 20 patients on Exjade therapy, for total of about 60 patients and a standard deviation of 25 obtained from the literature, the study will be able to predict an effect size of 13.

C. Study Procedure- N/A

D. Study Drugs- N/A

E. Medical Device- N/A

F. Study Questionnaires

A risk assessment questionnaire designed to assess several potential risk factors including age, sex, transportation costs, parental education, parental socioeconomic status, family support, appointment no show rate, medication self-management, developmental delay, forgetfulness, fear of adverse effects, lack of knowledge of benefits and sibling with SCD or other chronic disease. The questionnaires will also ask for attitudes toward text messaging as a method of reminding patients to take medications and opinions as to the content of the messages. Lastly there will an open ended question asking patients if they have found a specific method useful in improving adherence.

G. Study Subjects

Subjects will be included in the study if they have sickle cell disease, if they are between the ages of 10 and 17 years and are currently prescribed Hydroxyurea for greater than 18 months or Exjade. They will be excluded if they do not reside with a parent or legal guardian. Patients and parents must be able to speak English or Spanish.

H. Recruitment of Subjects

Using the Sickle Cell Clinic roster patients on Hydroxyurea will be selected based on fulfillment of inclusion criteria. They will be telephoned regarding the study and verbal consent will be obtained for study participation. A survey will then be conducted over the phone to assess risk factors, attitudes toward text messaging, preferred reminder text messaging content and alternative suggestions to improving medication adherence.

I. Confidentiality of Study Data

Confidentiality will be preserved by assigning de-identified subject numbers to surveys and medication refill data from pharmacies.

J. Potential Conflict of Interest-N/A

K. Location of the Study-N/A

L. Potential Risks

As the main focus of this study is to administer surveys and gather historical data on medication adherence

M. Potential Benefits

Patients who participate in this study have the potential to benefit because the knowledge gained in this study will inform the efforts of Community Health Workers that will work with the same patient population to address individual barriers to medication adherence. This will allow for a more targeted approach and an understanding of how patients would like text messaging to be used in their care.

N. Alternative Therapies- N/A

O. Compensation to Subjects

Patients will be mailed one Metrocard per family as a token of appreciation for participation in the study.

P. Costs to Subjects- N/A

Q. Minors as Research Subjects

R. Radiation or Radioactive Substances- N/A

References

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