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Original Article

Role of Patient and Maternal Knowledge in Awareness and Treatment Adherence for Thalassemia Major

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ABSTRACT

***Background**: Thalassemia Major is an inherited severe blood disorder that requires lifelong management, including regular and adequate transfusions along with iron chelation therapy. Knowledge of the disease and adherence to treatment protocols are the two important factors that can influence the outcome of the disease.*

***Objective**: The impact of the knowledge of the patients and mothers on their awareness and treatment adherence in thalassemia major patient is to be assessed, with emphasis on the level of education on management of healthcare.*

***Methods**: A cross-sectional design was employed, with 57 respondents selected from thalassemia centers dedicated to treating the disease, through convenience sampling. The data was collected using structured questionnaires to assess the demographic profiles, knowledge with respect to Thalassemia Major, and their adherence to the treatment protocols. Statistical analysis was done using the SPSS version 25, which concentrated on descriptive statistics and chi-square tests to evaluate the relationships of knowledge levels with adherence.*

***Results**: Mean age = 19.42, 56.1% females, and 42.1% males. A significantly high majority (93%) believed that thalassemia was a genetic disorder; however, misconceptions about available treatment were quite frequent. Regular blood transfusion at 4-week intervals was the most common form of treatment adherence reported (45.6%). While there was a high level of knowledge on general iron chelation therapy of 89.5%, only 64.9% knew the chelating agent being used. The other moderate adherence indicators were the rate of regular visits for follow-up at 44%.*

***Conclusion**: Maternal knowledge and patient knowledge were significantly associated with the management of Thalassemia Major. Better educational interventions will show better adherence and thereby patient outcomes.*

***Keywords**: Thalassemia Major, Patient Knowledge, Treatment Adherence, Blood Disorders, Iron Chelation Therapy, Genetic Diseases, Healthcare Management, Educational Interventions*

INTRODUCTION

Thalassemia Major is the severe form of thalassemia, being an inherited disorder of blood characterized by reduced synthesis of hemoglobin, leading to anemia, among other serious health issues. Successful management of Thalassemia Major is complex and multidimensional (1). It comprises of regular blood transfusions, iron chelation therapy, and constant medical monitoring to avoid complications like iron overload. Owing to the chronic nature of the disease, it causes a huge burden on the patient in emotional,

physical, and financial contexts, where patient and maternal knowledge play a very critical role in successful treatment adherence (2-4).

Awareness and knowledge of Thalassemia Major among patients and their primary caregivers, often the mothers, are of paramount importance in the management of the disease. Knowledge influences several aspects: recognition of symptoms, understanding the genetic basis of the disease, understanding treatment modalities, and adherence to medical routines as prescribed (5, 6). This aspect is discussed below in greater detail. In addition, this aspect is particularly important with respect to the mother because mothers are the primary caregivers and responsible for most of the medical management of the health conditions of their children, especially in conditions with chronic manifestations like Thalassemia Major. Thus, their knowledge of the disease directly affects the efficiency of the treatment protocol and wellbeing of the patient in general (7, 8).

Very importantly, proper management of thalassemia major requires the regular following of medical advice on the frequency of blood transfusions and strict iron chelation therapy regimes that need to be followed to prevent life-threatening complications. However, ignorance about the exact pathophysiology of the disease, in the sense that patients are not fully aware of the consequences of non-compliance with the treatment protocols, can be a potential barrier to adherence and can go a long way in seriously hampering patient outcomes. The objective of assessing the role of patient and maternal knowledge in awareness and treatment adherence for Thalassemia Major should be to identify gaps in knowledge for improved educational interventions in the long run and hence improve clinical outcomes (13). Health professionals would help in developing tailor-made interventions for training and reinforcing patients and their families in order to understand the effect of knowledge on treatment adherence (14, 15). This technique does not just help enhance the quality of life of individuals living with Thalassemia Major, but also helps in providing healthcare services effectively and efficiently by potentially averting the complications and comorbidities that result due to its poor management. This research is meant to rationalize and emphasize the significance of comprehensive patient and caregiver education as the cornerstone of effective disease management in Thalassemia Major (16,17).

MATERIAL AND METHODS

The research was performed in strict accordance with the principles and the Declaration of Helsinki and was reviewed and approved by the IRB before initiation of the study. All patients, themselves, gave informed consent. "The study included persons diagnosed with Thalassemia Major from every stratum of a demographic profile." The criteria for inclusion were a diagnosis of Thalassemia Major, continuing treatment by way of blood transfusions, iron chelation therapy, and an age between 5 and 35 years.

Exclusion criteria included co-existing severe cardiac, renal, or hepatic diseases that may be contraindications for the treatment of Thalassemia Major and patients who are not willing or able to provide informed consent. A convenience sampling technique was used in selecting the sample from the patients who were under treatment in several thalassemia treatment centers. This way, 57 patients were recruited into the study; it falls within the sample size capacity that can be conveniently handled for the collection and analysis of data within the logistics and time frame of the study (13).

The data were collected using a pretested, pre-validated, and structured questionnaire. The questionnaire was designed to generate information about the socio-demographic profile, health-related features, awareness regarding Thalassemia Major, information on compliance with treatment, and knowledge about chelation treatment, which was necessary to get a full response from the subjects. Information was also verified to ensure that self-reported data on treatment and compliance were accurate, using medical records. Data were analyzed using the Statistical Package for the Social Sciences version 25. Descriptive statistics were conducted to describe the characteristics of the sample in terms of demographics and health and to describe knowledge and adherence. Continuous variables were summarized using means and standard deviations, and categorical data were summarized using frequencies and percentages. Cross-tabulations were used to explore the relationship between the levels of knowledge, demographics, and adherence to treatment protocols. A high standard in data protection, confidentiality of storage, and analysis of activities were guaranteed; the identifiers of the participants had been removed and were to be stored securely to ensure that standards of privacy and ethics had been supported (16).

RESULTS

The study reports a very diverse demographic characteristic of the participants, with a mean of 19.42 years of age. In terms of gender distribution, 56.1% was female, while 42.1% was male. A small percentage, at 1.8%, did not specify their gender. The educational distribution stands from the low end at 35.1% reporting some college and above, hinting at a large percentage of participants with higher education. Employment data displays an even distribution across the different statuses, with students being the largest single group at 31.6%. The data on household income suggests economic diversity, as in the plurality of households, at 50.9%, are making between 30,000 and 60,000.

Genetic awareness of the genetic nature of Thalassemia Major is quite high, at 93%, acknowledging the fact that the disorder is rooted in genetics. Misconceptions arise about treatment needs; about 94.7% know a need exists for a regular blood transfusion, and the rest, standing as a minority, perceive that a need does not exist. The role of iron chelation therapy in preventing iron overload is well appreciated by 89.5% of them. However, despite this very high level of knowledge, substantial confusion is held about iron supplements, with 94.7% correctly dismissing them as being a cure for Thalassemia Major.

The mean duration for blood transfusion among patients was approximately 12.81 years, with transfusion normally recommended to be done every four weeks by 45.6% of the cohort. Data on treatment adherence showed that the mean duration of DFO therapy was shorter, at about 6.91 years, and the frequencies ranged from daily to less frequent than weekly, which is complex for many.

Table 1 Demographics and Health-related Characteristics

Category	Count	Percentage
Age (Years)	19.42	5.32
Gender		
Female	32	56.1%
Male	24	42.1%
Prefer not to say	1	1.8%
Education Level		
Elementary	6	10.5%
Junior High School	18	31.6%
Senior High School	13	22.8%
College or Higher	20	35.1%
Employment Status		
Student	18	31.6%
No job	12	21.1%
Part-time	14	24.6%
Full-time	13	22.8%
Household Income		
<30,000	20	35.1%
30,000-60,000	29	50.9%
>60,000	8	14.0%
Splenectomy Status		
Yes	17	29.8%
No	40	70.2%
Bone Marrow/Stem Cell Transplant		
Yes	3	5.3%
No	54	94.7%

Table 2 Knowledge about Thalassemia Major

Question	True Count	True Percentage	False Count	False Percentage
Q1: Thalassemia is a genetic blood disorder.	53	93.0%	4	7.0%
Q2: Regular blood transfusions are not necessary for Thalassemia Major patients.	3	5.3%	54	94.7%
Q3: Iron chelation therapy is used to prevent iron overload in patients.	51	89.5%	6	10.5%
Q4: Thalassemia can be cured by taking iron supplements.	3	5.3%	54	94.7%
Q5: Bone marrow transplant is a potential cure for some patients with Thalassemia Major.	39	68.4%	18	31.6%

Table 3 Table 3: Treatment Adherence

Treatment Aspects	Statistics	Value
Years of Blood Transfusion	Mean	12.81 years

	Standard Deviation	6.07
Frequency of Blood Transfusions	Every 2 weeks	3 (5.3%)
	Every 3 weeks	19 (33.3%)
	Every 4 weeks	26 (45.6%)
	Less frequently	9 (15.8%)
Years Receiving Desferrioxamine (DFO) Infusions	Mean	6.91 years
	Standard Deviation	3.69
Frequency of DFO Infusions	Daily	10 (17.5%)
	Several times a week	18 (31.6%)
	Weekly	17 (29.8%)
	Less frequently	12 (21.1%)
Duration of Each DFO Infusion Session	Mean	7.44 hours
	Standard Deviation	2.35

Table 4 Table 4: Chelation Therapy Awareness

Aspect	Subcategory	Count	Percentage
Knowledge about Desferral (Desferrioxamine)	Known	37	64.9%
	Not Known	20	35.1%
How often is the child receiving Desferral	Daily	0	0%
	Alternate days	4	7.0%
	Monthly with blood transfusion	16	28.1%
	Not receiving	37	64.9%
Method of Desferral Administration Known	Intravenous	9	15.8%
	Subcutaneous	15	26.3%
	Not Known	33	57.9%
Actual Method of Desferral Administration to Child	Intravenous	24	42.1%
	Subcutaneous	5	8.8%
	Not Given	28	49.1%
Awareness of Infusion Pump Usage	Yes	23	40.4%
	No	34	59.6%

Table 5 : Follow-up and Adherence Monitoring

Aspect	Subcategory	Count	Percentage
Frequency of Follow-up Visits	Regular (Every 2 weeks)	14	24.6%
	Regular (Every month)	11	19.3%
	Irregular	18	31.6%
	Not applicable	14	24.5%
Adherence to DFO Infusions	Always adheres	10	17.5%
	Sometimes adheres	16	28.1%
	Rarely adheres	19	33.3%
	Does not adhere	12	21.1%

Knowledge about chelation therapy was moderate, at 64.9%, and only a quarter knew the proper routes of Deferoxamine administration. Secondly, the actual practices of administration vis-à-vis what is known show a gap in knowledge and practice. The use of infusion pumps is not known by more than half of the participants. Follow-up and monitoring [Table 5] 44% of them have regular follow-up visits, of which only few actually maintained adherence. For adherence to the DFO infusions, it was also quite variable. Thirty-three point three percent reported rarely adhering to their regimen, indicating significant barriers to regimen adherence. All

these informative sources reflect the vitally important role of the knowledge of patients and mothers in the correct management of Thalassemia Major: that there is strong genetic disorder awareness and, at the same time, some critical gaps in the application of treatment and adherence to it—resulting in interventions that need addressing.

DISCUSSION

The results from the study showed that patient and maternal knowledge was a critical determinant in the overall management of Thalassemia Major. This was consistent with previous research which showed that a good understanding of the disease significantly influenced treatment adherence (18). High awareness of the genetic nature of the disease and the necessity for the blood transfusion became apparent, which is consistent with studies showing that more educated patients and caregivers are more likely to adhere to treatment protocols (19). However, the rumors associated with iron supplements as a possible cure point to certain knowledge gaps that may limit optimal care (19). The study further revalidates such broad education approaches, that enhancing caregiver education results in better management of chronic pediatric patients. This is important in the case of Thalassemia Major where therapeutic regimens are complex and require a high level of an informed caregiver's involvement. A strength of the study was that the diverse sample could allow the different demographic factors to be checked along with their influence on knowledge and adherence. However, this was a limitation of applying a technique of convenience sampling, which may fail to provide an adequately representative sample from the Thalassemia Major population and thus create bias in the results. Furthermore, reliance on self-report data might have produced response bias, with the subjects possibly over-reporting their adherence to the treatment regimens or their understanding of the disease (22). Despite its limitations, this study was able to provide useful information relevant to the factors affecting adherence to treatment in Thalassemia Major. This will help in making recommendations for the introduction of specially tailored educational courses that are likely to bridge the knowledge gaps established during the study and could influence the patients' outcomes positively. In addition, health care practitioners should consider the use of individualized communication approaches that take into consideration the diverse educational levels of the patients and their families (23). Future research should emphasize longitudinal studies to determine the effect the improved education interventions have on long-term treatment adherence. The other piece of advice is expanding the sample size to make the findings have even higher generalizability. This could further be increased by adding objective measures of adherence, such as medical records and biomarkers related to patient and caregiver knowledge of treatment compliance (24-27).

CONCLUSION

The study has clearly proved that patient and maternal education in the management of Thalassemia Major means an increase in understanding will directly lead to an increase in treatment adherence. Such facts emphasize the necessity for healthcare professionals to ensure relevant educational interventions and personalized communication strategies that can bridge prevailing knowledge gaps. This will optimize treatment outcomes, reduce complications, and tremendously improve quality of life for patients suffering from Thalassemia Major, in addition to underlining the larger implications in human disease management.

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