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Monitoring Of Clinical Presentations, Types, Management and Outcomes in Thalassemia Patients

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Abstract:
Background*: Thalassaemia is a autosomal recessive hereditary chronic blood disorder that does not allow the body to produce enough haemoglobin and red blood cells.The primary management is blood transfusions which leads to complications. Our study aims to evaluate the clinical presentations and complications, observe the prescribing pattern , disease to understand and improve the knowledge of study participants.*
Materials and Methods*: This is a prospective observational study, was conducted in the pediatrics department of tertiary care hospital(GGH-RIMS) in Kadapa, Andhra Pradesh, India, during the time period of 6 months, A total of 50 patients, both transfusion dependent and non-transfusion dependent thalassemia patients of all age groups involed in the study by taking informed consent form .*
Results*: In our study, out of 50 patients , 48 (96%) patients were prescribed with Deferasirox(20 – 40 mg/kg), 41 (82%) patients were prescribed with Deferiprone(25mg/kg) , 5 (10%) patients were prescribed with Hydroxy Urea(20-30 mg/kg) , 50(100%) patients were prescribed with folic acid(2.5 – 5 mg), The knowledge of patient and patient care takers was inadequate in pre-education and was improved in post education.*
Conclusion: *Our study observed that blood transfusion was primary therapy for thalassemia major, complications were decreased pre-transfusion Hb, RBC ,altered liver enzymes ,and there is need for knowledge improvement about disease among patient and patient care takers .*
Key Words*: Thalassemia major , Deferasirox , Deferiprone , Blood transfusion.*

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I. Introduction

Thalassemia (thal) is an autosomal recessive hereditary chronic haemolytic anemia due to a partial or complete deficiency in the synthesis of α-globin chains (α-thal) or β-globin chains (β-thal) which compose the major adult hemoglobin (HbA), a tetramer of α2β2. Clinically classified as Thalassemia major (TM) Thalassemia intermedia(TI) Thalassemia minor .TM has Severe anemia presenting early in life and requiring life long blood transfusions and iron chelation. TI has mild to moderately severe anemia, requiring no blood transfusions to occasional and frequent blood transfusions, Minor is asymptomatic with mild anemia and a heterozygous condition (trait) of thalassemia, requires no transfusion, but genetic counseling. Clinical presentations are Severe anemia (Hb <7 g/dL), pallor, jaundice, irritability, feeding problems, failure to thrive, skeletal deformities, delayed growth and development ,abdominal enlargement owing to progressive splenomegaly and hepatomegaly, or recurrent episodes of infection. Both TDT and NTDT share the pathological cascade of α-to-β globin chain imbalance, ineffective erythropoiesis .Most patients with thalassemia are diagnosed before two years of age by using Hb electrophoresis, Hb HPLC, Full blood counts show low haemoglobin (Hb), MCV and MCH . Managed with transfusion ,Iron Chelation Therapy , Haematopoietic Stem Cell Transplantation (HSCT), gene therapy , splenectomy.

II. Material And Methods

This study was carried out on patients of Department of paediatrics at Government general Hospital (RIMS), Kadapa, Andhra Pradesh, India, from January 2021 to June 2021. A total 50 subjects (both male and females) of all age groups were included in this study.
Study Design: Prospective observational study

Study Location: Government General Hospital - RIMS, Kadapa, Andhra Pradesh, India.
Study Duration: 6 months, from January 2021 to June 2021.
Sample size: 50 patients.
Subjects & selection method: we selected both transfusion dependent and non-transfusion dependent thalassemia patients of all age groups involved in the study by taking informed consent form .

Inclusion criteria:
1. Thalassemia Patients who were willing to participate in the study.
2. Thalassemia Patients of all the age groups irrespective of gender.
3. Thalassemia patients who were taking chelation therapy
4. Thalassemia Patients who were having complications.
5. Thalassemia Patients who comes for regular follow-ups.
6. Known case of thalassemia who were under treatment

Exclusion criteria:
1. Thalassemia Patients who were not willing to participate in the study.
2. Thalassemia Patients who were not able to explain about the disease condition /treatment.
3. Thalassemia Patients who were pregnant women.
4. Patients who were not confirmed with thalassemia.

Procedure methodology:
We have identified known thalassemia patients with proper diagnosis.
After explaining about the study we have included them in to the study by using written informed consent form
On visit basis, we have collected demographic details, clinical manifestations, laboratory and radiological reports of selected patients after collecting medical history and have recorded the data.
According to the table, we have assessed the patients to know the progress and outcomes of the disease.

Table -1: Study assessment parameters

parameters	Base line	1st follow up	2nd follow up
Height	✓	✓	✓
Weight	✓	✓	✓
Hb	✓	✓	✓
RBC	✓	✓	✓
WBC	✓	✓	✓
MCH	✓	✓	✓
MCV	✓	✓	✓
MCHC	✓	✓	✓
Platelet count	✓	✓	✓
Serum ferritin	✓	✓	✓
AST	✓		
ALT	✓	✓	✓
Bilirubin	✓		
Albumin	✓		
Creatinine	✓	✓	✓
TSH/T3/T4	✓		
Calcium	✓		
RBS	✓		
Vit-D	✓		

Statistical analysis:
We have used descriptive statistics like percentage, mean, standard deviation for analysing the different parametrs and the collected data analysed by usingMS excel and Graphpad prism .

III. Results:

Among 50 patients 30 were male and 20 were female . The most of the patients were between the age group of 1-10 years (54 %) followed by 11 – 20 years (38 %) and 21-30 (8%) . Majority of patients were diagnosed with thalassemia major (88 %) followed by thamlassemia intermedia (12%) .

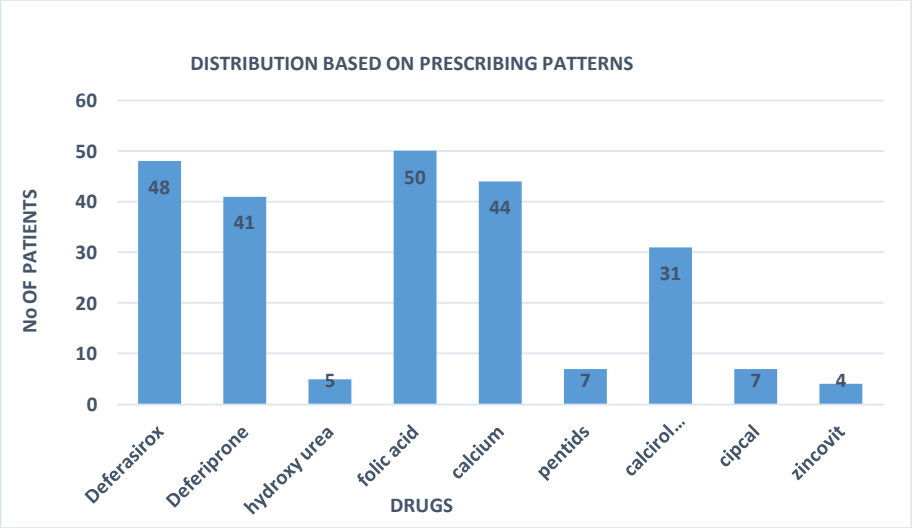

Figure 1: Distribution based on prescribing patterns

The most commonly prescribed monotherpay was Deferasirox (8%) and Hydroxy urea (4%), combination therapy was Deferasirox and Deferiprone (83%) followed by Deferasirox and Hydoxy urea (6%) .

Table 2: Average values of the assessing paraemeters

s.no	parameter	baseline	1st follow up	2nd follow up
1.	Hb	9.02	9.2	9.05
2.	Rbc	3.59	3.67	3.50
3.	MCV	81.5	81.14	80.15
4.	MCH	25.08	25.24	25.89
5.	platelets	4.17	3.75	3.02
6.	WBC	9278	9598	8873.8
7.	Ferritin	4440	4528	4218.3
8.	ALP	188.9	188.08	195.6
9.	SGOT	72.1	62.16	58.06
10.	SGPT	72.66	56.38	58.6
11.	Total bilirubin	1.456	1.24	1.368
12.	Serum creatinine	0.53	0.522	0.50
13.	Calcium	9.48	10.03	9.79
14.	Weight	25.206	26.722	28.362
15.	Height	122.3	124.34	127.31

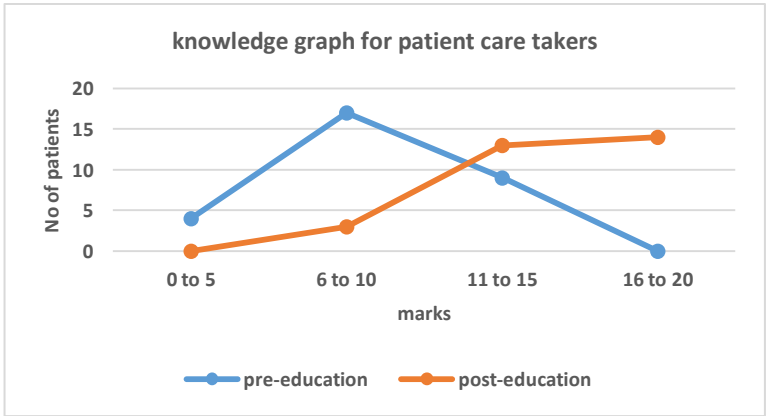

Figure 2: Distribution based on knowledge of patient care takers

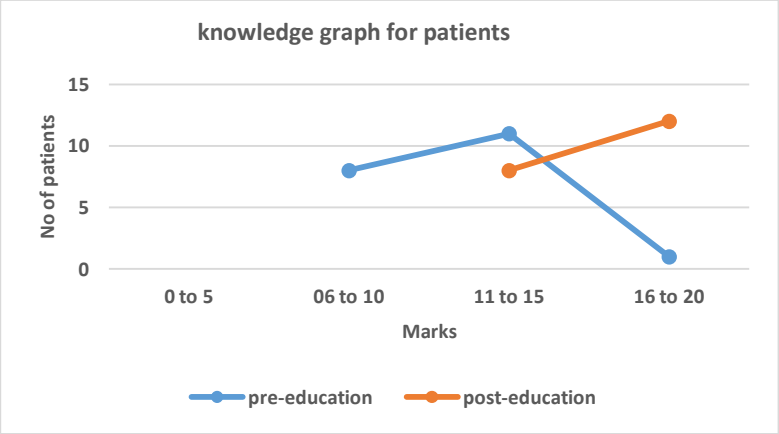

Figure 3: Distribution based on knowledge of patients

IV. Discussion

In our study we have observed complications due to the increased number of blood transfusions were high ratio of decreased pre-transfusion Hb, RBC, Altered liver enzymes, hepatomegaly, splenomegaly, growth retardation, hyper pigmentation and haemolytic facies.

Wasuwit W et.al(2) conducted a study, according to this study the lower pre transfusion Hb levels was associated with frequent complications in both TDT and NTDT patients. Optimal blood transfusion and effective iron chelation should be used to prevent these complications.

M.Mansour et.al(3) conducted a study ,according to this study 2.9% of patients were non anemic,2.9% were mild anemic,61.8% were moderate anemic, 32.4% were severe anemic.79.4% patients had low level of MCV and 20.6% had normal MCV.58.8% of patients had abnormally high WBC levels, 38.2% had normal WBC levels, 2.9% had low level WBC level. The ferritin levels of 77% of patients were greater than 2500ng/ml, 17% had level of 1000-2000ng/ml and 58% had equal to 1000ng/ml.

In our study out of 50 patients, 96% received iron chelation among them 14% received mono chelation therapy with DFX(20-40mg/kg/day). 82% received combination therapy with DFX(20-40mg/kg/day) and DFO(25mg/kg),10% received Hydroxyurea(20-30mg/kg)and also received folic acid, calcium, calcirol sachets, zincovit ,penicilins, cipcal.

Mohammad Reza Bordbar et.al(4) conducted a study , according to this study the patients were treated with hydroxyurea with mean dose of 10.5mg/kg for a duration of 8 months and their Hb levels were compared before and after HU treatment. The mean volume of blood transfusion decreased significantly following HU treatment so, concluded that HU can be safely used in some TDT patients to decrease their transfusion needs.

Tirin B et.al(5) conducted a study, according to this study 83% was prescribed with Deferasirox monotherapy(20-30mg/kg/day), 11% of patients was prescribed with combination of Deferasirox(20-30mg/kg/day) and Deferiprone(25mg/kg/day), 5% of patients were undergone splenectomy and also 16 DRPs and 15 medication errors were identified .

Sunil G et.al(6) conducted a study, according to this study patients were divided into 3 groups who received Deferiprone(75mg/kg/day) monotherapy, Deferasirox(30mg/kg/day) monotherapy and combination of DFP and DFX. After 12 months serum ferritin measured, there is a significant fall im mean ferritin levels of combination therapy compared to monotherapy.

According to the survey of our study that the scores of patient and parents regarding disease knowledge and treatment are satisfactory after educating them when compared to the scores of pre-education.

Ya-ling lee et.al(8) conducted a study , according to this study the current knowledge of patient is insufficient as their scores are very low, and there is a need to enhance patients education about thalassemia major.

Mohammad Azmi che Mat et.al(9) conducted a study, according to this study there is a need to improve the knowledge of patients and patient care takers regarding disease, treatment, and its complications because the knowledge score among patients and parents remains unsatisfactory in this study.

V. Conclusion

In this study we observed the prescribing pattern of drugs among thalassemia patients, T.Deferasirox, T.Deferiprone, T.folic acid and T. calcium was most commonly prescribed in thalassemia patients. We also observed that most prescribed combination therapy of iron chelators was T. Deferasirox and T. Defiriprone . This study demonstrates that primary therapy for thalassemia major was blood transfusion.

Complications observed due to the increased number of blood transfusions were high ratio of decreased pre-transfusion Hb, RBC, Altered liver enzymes, hepatomegaly, splenomegaly, growth retardation, hyper pigmentation and haemolytic facies.

We observed that the current knowledge of patients and patient care takers about disease was insufficient. Therefore the knowledge need to be improved among the patients and patient care takers. Health care providers need to enhance proper education and patient counselling with respect to disease, etiology, treatment and complications related to iron overload and also selfcare for the early identification and management of complications

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