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A Descriptive Study To Assess Endocrine Outcomes And Medication Adherence Among Children With Transfusion-Dependent B-Thalassemia Major Receiving Combination Oral Iron Chelation Therapy In Selected Hospitals Of Jammu

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ABSTRACT Background

Transfusion-dependent β-thalassemia major (TDT) is a severe inherited hemoglobin disorder requiring lifelong blood transfusions, which frequently result in iron overload and endocrine complications. Despite advances in combination oral iron chelation therapy, endocrine dysfunction remains a major concern. Medication adherence plays a crucial role in minimizing iron overload and improving clinical outcomes. Regular assessment of endocrine status and treatment adherence is therefore essential for optimizing long-term management.

Objectives

1. To assess the demographic and clinical profile of children with transfusion-dependent β-thalassemia major receiving combination oral iron chelation therapy.
2. To assess endocrine outcomes and medication adherence among children with transfusion-dependent β-thalassemia major.
3. To determine the association between endocrine outcomes and selected demographic and clinical variables.
4. To determine the relationship between medication adherence and endocrine outcomes.

Methods

A quantitative descriptive cross-sectional study was conducted among 150 children with transfusion-dependent β-thalassemia major receiving combination oral iron chelation therapy at selected hospitals in Jammu. Participants were selected using a consecutive samplingtechnique. Data were collected using a structured assessment tool comprising demographic and clinical variables, endocrine assessment, and medication adherence. Descriptive and inferential statistics were used for data analysis.

Results

Of the 150 participants, 58.7% were males and 41.3% were females, with a mean age of 10.9

± 3.2 years. Elevated serum ferritin levels were observed in 58.0%, abnormal fasting blood sugar in 18.0%, abnormal thyroid-stimulating hormone levels in 24.7%, growth retardation in 32.0%, and delayed puberty in 21.3% of age-eligible children. Good medication adherence was reported in 56.0% of participants. Medication adherence was significantly associated with improved endocrine outcomes ($p < 0.05$).

Conclusion

Endocrine abnormalities remain common among children with transfusion-dependent β-thalassemia major despite receiving combination oral iron chelation therapy. Better medication adherence was associated with improved endocrine outcomes, emphasizing the importance of regular monitoring and continuous nursing support.

Keywords: Transfusion-dependent β-thalassemia major, endocrine outcomes, medication adherence, oral iron chelation therapy, pediatric nursing.

INTRODUCTION

Transfusion-dependent β-thalassemia major (TDT) is a severe inherited hemoglobin disorder characterized by defective β-globin chain synthesis, resulting in chronic anemia that necessitates lifelong regular blood transfusions. Although advances in transfusion practices and iron chelation therapy have significantly improved survival, repeated transfusions lead to progressive iron overload, predisposing children to endocrine complications such as growth retardation, delayed puberty, hypothyroidism, and impaired glucose metabolism. These complications adversely affect growth, development, and quality of life.

Combination oral iron chelation therapy has improved the management of transfusional iron overload; however, treatment outcomes largely depend on medication adherence and regular clinical monitoring. Early identification of endocrine abnormalities and adherence to therapy are essential for preventing long-term complications. Therefore, the present study was undertaken to assess endocrine outcomes and medication adherence among children with transfusion-dependent β-thalassemia major receiving combination oral iron chelation therapy in selected hospitals of Jammu.

OBJECTIVES

1. To assess the demographic and clinical profile of children with transfusion-dependent β-thalassemia major receiving combination oral iron chelation therapy.
2. To assess endocrine outcomes and medication adherence among children with transfusion-dependent β-thalassemia major.
3. To determine the association between endocrine outcomes and selected demographic and clinical variables.
4. To determine the relationship between medication adherence and endocrine outcomes.

HYPOTHESIS

H_0: There will be no statistically significant association or relationship between endocrine outcomes, medication adherence, and selected demographic and clinical variables among children with transfusion-dependent β-thalassemia major receiving combination oral iron chelation therapy.

METHODOLOGY Research Design

A quantitative descriptive cross-sectional research design was adopted to assess endocrine outcomes and medication adherence among children with transfusion-dependent β-thalassemia major receiving combination oral iron chelation therapy.

Setting and Sample

The study was conducted in the Pediatric Hematology Departments of selected tertiary care hospitals in Jammu. A total of 150 children diagnosed with transfusion-dependent β-thalassemia major and receiving combination oral iron chelation therapy were selected using a consecutive sampling technique.

Eligibility Criteria

Children aged 5–18 years with confirmed transfusion-dependent β-thalassemia major, receiving regular blood transfusions and combination oral iron chelation therapy for at least six months, were included in the study. Children with congenital endocrine disorders, a history of bone marrow transplantation, or critical illness were excluded.

Description and Validation of the Tool

Data were collected using a structured tool comprising three sections: Part I included demographic and clinical variables, Part II assessed endocrine outcomes (serum ferritin, fasting blood sugar, thyroid-stimulating hormone, growth assessment, and pubertal status), and Part III assessed medication adherence. The tool was validated by experts in Pediatric Nursing, Pediatric Hematology, Pediatric Endocrinology, and Biostatistics. Reliability was established through pilot testing using Cronbach's alpha coefficient.

Data Collection Procedure

After obtaining ethical approval and institutional permission, written informed consent was obtained from parents or legal guardians. Demographic and clinical data were collected through interviews and

medical records, while endocrine outcomes and medication adherence were assessed using the structured tool during scheduled hospital visits.

Ethical Considerations

Ethical clearance was obtained from the Institutional Ethics Committee before commencement of the study. Confidentiality was maintained throughout the study, and participation was voluntary.

Data Analysis

Data were analyzed using the Statistical Package for the Social Sciences (SPSS). Descriptive statistics (frequency, percentage, mean, and standard deviation) summarized the data, while the Chi-square test and Pearson's correlation analysis were used to determine associations and relationships. A p-value < 0.05 was considered statistically significant.

RESULTS

Table 1. Demographic and Clinical Profile of Children with Transfusion-Dependent β-Thalassemia Major (N = 150)

Variable	Category	n	%
Age (Years)	5–8	38	25.3
	9–12	57	38.0
	13–18	55	36.7
Gender	Male	88	58.7
	Female	62	41.3
Age at Diagnosis	<1 year	41	27.3
	1–2 years	82	54.7
	>2 years	27	18.0
Blood Transfusions/Year	8–12	46	30.7
	13–16	72	48.0
	>16	32	21.3

Most children (38.0%) were aged **9–12 years**, **58.7%** were males, and **48.0%** received **13–16 blood transfusions annually**.

Table 2. Distribution of Endocrine Outcomes and Medication Adherence among Children with Transfusion-Dependent β-Thalassemia Major (N = 150)

Variable	Category	Frequency (n)	Percentage (%)
Serum Ferritin	≤2500 ng/mL	63	42.0
	>2500 ng/mL	87	58.0
Fasting Blood Sugar (FBS)	Normal	123	82.0
	Elevated	27	18.0
Thyroid-Stimulating Hormone (TSH)	Normal	113	75.3
	Elevated	37	24.7
Growth Assessment	Normal Growth	102	68.0
	Growth Retardation	48	32.0
Pubertal Status*	Normal	59	78.7
	Delayed Puberty	16	21.3
Medication Adherence	Poor	21	14.0
	Moderate	45	30.0
	Good	84	56.0

***Pubertal status was assessed only among age-eligible participants (n = 75).**

Elevated serum ferritin levels were observed in 58.0% of participants, while 18.0% had elevated fasting blood sugar and 24.7% had abnormal TSH levels. Growth retardation was identified in 32.0% of children, and delayed puberty was observed in 21.3% of age-eligible participants. More than half of the participants (56.0%) demonstrated good medication adherence.

Table 3. Association between Endocrine Outcomes and Selected Variables (N = 150)

Variable	χ^2	df	p-value
Age	9.64	2	**0.008***
Gender	1.82	1	0.177
Duration of Illness	8.15	2	**0.017***
Annual Blood Transfusions	10.86	2	**0.004***
Duration of Chelation Therapy	6.52	2	**0.038***

*$p < 0.05$. Endocrine outcomes were significantly associated with age, duration of illness, annual blood transfusions, and duration of chelation therapy.

Table 4. Correlation between Medication Adherence and Endocrine Outcomes (N = 150)

Variables	r	p-value
Medication Adherence & Serum Ferritin	−0.61	**<0.001***
Medication Adherence & Fasting Blood Sugar	−0.39	**<0.001***
Medication Adherence & TSH	−0.36	**<0.001***

*Correlation is significant at $p < 0.001$. Medication adherence showed a significant negative correlation with serum ferritin, fasting blood sugar, and TSH levels, indicating that better adherence was associated with more favourable endocrine outcomes.

DISCUSSION

The present study assessed endocrine outcomes and medication adherence among children with transfusion-dependent β-thalassemia major receiving combination oral iron chelation therapy. The findings indicated that endocrine abnormalities, particularly elevated serum ferritin levels, thyroid dysfunction, impaired glucose metabolism, and growth retardation, remain common despite ongoing treatment. Better medication adherence was significantly associated with improved endocrine outcomes, highlighting the importance of regular monitoring, adherence counselling, and comprehensive nursing care to optimize long-term disease management.

LIMITATIONS

- The study was conducted in selected tertiary care hospitals of Jammu, limiting the generalizability of the findings.
- The cross-sectional design did not permit the establishment of causal relationships.

CONCLUSION

The study demonstrated that endocrine abnormalities remain prevalent among children with transfusion-dependent β-thalassemia major receiving combination oral iron chelation therapy. Better medication adherence was associated with more favourable endocrine outcomes, emphasizing the need for regular endocrine assessment and continuous nursing support to improve long-term patient outcomes.

RECOMMENDATIONS

1. Regular endocrine screening and medication adherence assessment should be integrated into routine follow-up care.
2. Larger multicentre studies are recommended to further evaluate endocrine outcomes among children with transfusion-dependent β-thalassemia major.

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