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**Original Research Article**

# Evaluation of health-related quality of life among transfusion dependent thalassemia major children aged 6-18 years

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## ABSTRACT

**Background:** Children with transfusion-dependent thalassemia major (TDTM) are vulnerable to issues in various aspects of psychological, physical, social, emotional, communication and educational which results in diminished health-related quality of life (HRQOL). Aim was to evaluate HRQOL and corelation with sociodemographic factors among TDTM children between the aged 6-18 years.
**Methods:** This cross-sectional study involved 69 children with TDTM aged 6-18 years by using validated paediatric QOL inventory version 4.0 (Peds QL) to assess HRQOL, which includes four domains 1) physical functioning (8 items) 2) emotional functioning (5 items), 3) social functioning (5 items) and 4) school functioning (5 items) and were rated on a five-point Likert scale 0 to 4. The items on the Peds QoL were reverse scored and converted to a 0-100 scale. Higher scores signify better HRQOL. The data were analysed statistically.
**Results:** The mean age of the cohort was 11.58±3.71 years while the majority were male (72.46%). Physical and school functioning scores were significantly lower in the 16-18 years group (p=0.0094) and (p=0.0413). There was no statistically significant difference in HRQOL between sexes. Educational status showed significantly higher total HRQOL scores in 7$^{th}$ 9$^{th}$ grade (p=0.0481). The frequency of blood transfusion did not significantly impact HRQOL. Deferasirox users reported higher total HRQOL scores.
**Conclusions:** HRQOL was significantly impacted among TDTM children. Patients not receiving chelation therapy had recorded the lowest HRQOL scores, highlighting the importance of effective chelation in managing iron overload and improving HRQOL.

**Keywords:** Children, Health related quality of life, Transfusion dependent, Thalassemia major

## INTRODUCTION

The quality of life (QOL) includes an individual's physical health, psychological state, level of independence, social relationships, personal beliefs, and their connections to significant features of the environment.[1] An evaluation of the HRQOL quantifies the impacts of a condition and its management on a individual's physical and mental health.[2,3] Transfusion dependent thalassemia major (TDTM) is characterized by partial or complete lack of alpha or beta globin chain production and needs regular blood transfusion.[4] As the morbidity and mortality in children suffering from TDTM has significantly reduced over the past decade because of modern medical management the importance of QOL should be emphasized in them. These children are vulnerable to multiple problems in various aspects of psychological, physical, social, emotional, communication and educational functioning, which leads to impaired HRQOL.[5]

A proper understanding of these factors associated with HRQOL in children with thalassemia would improve clinical counselling and treatment outcomes. Furthermore, it may assist health policymakers to modify or enhance long-term medical facilities in these children. The aim of this study was to evaluate HRQOL and its correlation with sociodemographic factors among TDTM children between the age group of 6-18 years.

## METHODS

The present cross-sectional observational study was conducted in the department of paediatrics at Dr. D. Y. Patil medical college, hospital and research institute, thalassemia day care centre, Kolhapur between 5 December 2022 and 5 May 2024 after institutional ethical committee approval (IEC-42/2022-23). Total of 69 children who were diagnosed with TDTM between the ages of 6-18 years and whose parents consented to participate in this study were included. TDTM children with other disease elements like acute renal failure, congestive cardiac failure, neurological disease etc or with any acute life events (loss of a loved one, acute illness requiring hospitalisation, change in school/residence etc) were excluded.

The sample size (n) was calculated using the Slovin formula which is as follows.

Sample size=$n=N/1+Ne^2$

N=Population size, e=Margin of error

By considering margin of error=e=10%=0.10 for N=214 patients, we get

$N=214/1+214\times(0.1)^2$

N=214/3.14=68.15

Minimum sample size required was 69.

This study used the validated paediatric QOL inventory version 4.0 (Peds QL) to measure HRQOL[6]. It consisted of 1) physical functioning (8 items), 2) emotional functioning (5 items), 3) social functioning (5 items) and 4) school functioning (5 items). A structured questionnaire was given to the child in printed format in the local language or the language which the child can understand well. If the child could not read, the question was read by the interviewer to the child and child was asked for the preference and, the response was marked. All questions were answered based on the self-evaluated status from the past two weeks prior to enrolment and were rated on a five-point Likert scale of 0-4.

If any question in the questionnaire disturbed the child or caused discomfort, he was referred to a psychologist for counselling after informing the parents.

The items on the Peds QoL generic core scales were reverse scored and transformed to 0-100 scale. Higher scores indicate better HRQL: 0 (“never”)=100, 1 (“almost never”)=75, 2 (“sometimes”)=50, 3 (“often”)=25, and 4 (“Almost Always”)=0. Scale scores were computed as the sum of the items divided by the number of items answered (to address the missing data).

If more than 50% of items were missing, the Scale Score would not be computed. The psychosocial health summary score represents the average score on the emotional, social and school functioning scales. The physical health summary score corresponds to the physical functioning scale score. The total scale score is the mean of all items.

### *Statistical analysis*

Categorical variables were analysed using IBM SPSS version 20 to determine their frequencies and percentages, and with results visualized using pie charts to show their distribution. Continuous variables were assessed for measures of central tendency (mean) and spread (standard deviation), and the data were graphically represented.

## RESULTS

In this study, a total 69 TDTM children were included with a mean age of 11.58±3.71 years. The highest percentage 44.93% (n=31) of children were between six and ten years old followed by 33.33% (n=23) in the 11-15 years, and 21.74% (n=15) in 16-18 years age groups, with the majority 72.46% (n=50) being male.

The highest percentage of children, 39.13% (n=27), were in first to six grades while, 33.33% (n=23) and 27.54% (n=19) were in seventh to ninth, and tenth to twelfth grade respectively.

The frequency of blood transfusions among the study subjects varied. The most common interval for blood transfusions was 21 days, occurring in 42.03% (n=29) of the cases while the least common interval was monthly, observed in 26.09% (n=18) of children. The type of chelation therapy utilized demonstrated a clear preference of 84.06% (n=58) for deferasirox.

The comparison of HRQOL scores with age, sex, educational status, frequency of blood transfusion and type of chelation agent is illustrated in Table 1.

Overall, HRQOL was significantly impacted in TDTM children. The frequency of blood transfusions did not significantly affect overall HRQOL whereas Deferasirox was associated with higher HRQOL scores than Deferoxamine and Deferiprone. Also, Children who did not receive chelation therapy had the lowest HRQOL scores.

**Table 1: The comparison of HRQOL score with age, sex, educational status, frequency of blood transfusions and type of chelation agent HRQOL.**

| Parameters | | HRQOL | | | | |
|---|---|---|---|---|---|---|
| | | Physical | Emotional | Social | Educational | Total |
| Age (in years) | 6-10 | 57.88±12.21 | 56.83±12.87 | 63.35±12.41 | 53.35±13.09 | 57.85±7.88 |
| | 11-15 | 58.58±12.37 | 61.30±13.16 | 62.82±12.38 | 60.43±14.13 | 60.78±8.14 |
| | ≥15-18 | 47.53±12.22 | 60.66±12.90 | 57.33±12.56 | 49.6±12.79 | 53.78±7.97 |
| | P value | 0.0094 | 0.4019 | 0.2485 | 0.0413 | 0.0252 |
| Gender | Male | 56.16±12.24 | 58.54±12.14 | 61.98±12.87 | 55.98±14.38 | 57.38±8.14 |
| | Female | 55.08±12.43 | 60.78±14.83 | 61.57±11.43 | 52.05±12.22 | 58.16±7.88 |
| | P value | 0.7454 | 0.5206 | 0.9056 | 0.2959 | 0.7064 |
| Education (class grade) | $1^{st}$-$6^{th}$ | 57.49±12.51 | 55.81±15.27 | 59.96±11.21 | 54.22±13.03 | 56.87±7.88 |
| | $7^{th}$-$9^{th}$ | 59.37±10.57 | 62.39±12.14 | 66.73±13.53 | 56.08±14.13 | 61.14±8.01 |
| | $10^{th}$-$12^{th}$ | 49.29±11.65 | 60 ±8.81 | 58.68±11.40 | 54.42±15.22 | 55.60±7.96 |
| | P value | 0.0172 | 0.1883 | 0.0642 | 0.0413 | 0.0481 |
| Blood transfusion frequency | 15 days | 55.02±11.48 | 60.68±11.67 | 62.90±13.55 | 54.77±13.04 | 58.35±7.97 |
| | 21 days | 58.85±11.92 | 59.20±10.72 | 63.62±11.64 | 56.06±13.57 | 58.94±7.88 |
| | Monthly | 52.07±12.97 | 57.22 ±17.25 | 57.22 ±17.25 | 56.38±15.79 | 55.87±7.94 |
| | P value | 0.1687 | 0.3511 | 0.2643 | 0.1533 | 0.4193 |
| Type of chelation | Deferasirox | 57.03±11.76 | 60.53±12.95 | 63.48±12.50 | 55.13±13.93 | 59.04±7.94 |
| | Deferoxamine | 47.62±10.79 | 54±12.87 | 56±12.41 | 54±13.84 | 53.90±7.88 |
| | Deferiprone | 35±0 | 55±0 | 35±0 | 70±0 | 48.75±0.00 |
| | Nil | 31.86±31.72 | 41.66±12.35 | 48.33±12.01 | 46.66±13.74 | 47.80±8.38 |
| | P value | <0.0001 | <0.05 | <0.05 | <0.05 | 0.0315 |

The correlation analyses indicated a very weak negative correlation between age and HRQOL (r=-0.09, p=0.4459), no significant correlation with gender (r=-0.04, p=0.7133), a very weak negative correlation with education (r=-0.03, p=0.7705), and a very weak negative correlation with the frequency of blood transfusion (r=-0.11, p=0.3563). A moderate negative correlation was identified between HRQOL and type of chelation therapy, which was statistically significant (r=-0.31, p=0.0074).

## DISCUSSION

TDTM may have negatively affected HRQOL due to multiple reasons. The physical changes caused by the illness, deformed facial features, and stunted growth, can affect a child's self-esteem and confidence, making them feel different or stigmatized. These changes can also impact child's ability to function normally. Frequent hospital visits for blood transfusions, extended periods spent receiving iron chelation therapy infusions, and fatigue from anaemia can disrupt a child's routine and attendance at school. This can further complicate their daily lives and educational experience.[7,8] The main aim was to evaluate the HRQOL using the Peds QL questionnaire. Additionally, this study aimed to identify and correlate socio-demographic variables such as age, sex, education level, type of chelation, and frequency of blood transfusions with HRQOL among these patients.

This study revealed significant differences in HRQOL across various age groups. The physical functioning scores were significantly higher in the younger age groups (6-10 years and 11-15 years) compared to the older age group (16-18 years). This decline in physical functioning scores with age could be attributed to the cumulative burden of chronic disease and their complications, which may become more pronounced as children grow older. Emotional, social, and school functioning scores did not show significant differences across age groups, though school functioning was notably better in the 11-15 years group compared to the other age groups. The overall HRQOL scores also indicated a significant decline in the older age group. The weak negative correlation between HRQOL and age, although not statistically significant, suggests a trend where older children may experience a lower QOL, potentially due to increased challenges related to their disease. However, Mardhiyah et al observed that QOL increased with age, which is consistent with our present study.[9] Additionally, they found a significant correlation between age and HRQOL in children, which stands in contrasts with our study findings. The differences may arise from variations in study populations, inclusion criteria and study design type. Both, sexes reported similar HRQOL scores, indicating that sex does not play a significant role in determining the HRQOL. The correlation analysis also supported this finding and was also comparable with study conducted by Lam et al while Mardhiyah et al observed that girls had higher scores of QOL than boys in physical and emotional health.[9,10]

The study found that education status had significantly impacted specific domains of HRQOL The education status showed significant differences, with higher scores in physical functioning in seventh to ninth grade as compared to other grades (p=0.0172) and significantly greater total HRQOL scores in this grade group as well

(p=0.0481). This could be due to a variety of factors, including the possibility that older children in lower grades might experience feelings of academic inadequacy or social stigma. However, the correlation between HRQOL and education was very weak and not statistically significant. This indicates that while education status may influence certain HRQOL domains, it is not a strong overall determinant and similar observations were made by Mardhiyah et al and Hakeem et al.[9,11]

Interestingly the frequency of blood transfusion did not significantly impact HRQOL if they were done regularly based on pre-transfusion Hb levels. This suggests that although regular blood transfusions are a critical part of managing TDBT, interval between transfusions does not significantly impact the overall QOL as measured by the Peds QL. The weak negative correlation between HRQOL and transfusion frequency, although not statistically significant, indicates that more frequent transfusions do not necessarily lower QOL. Our study findings were comparable to those of Mardhiyah et al.[9] This may result from regular transfusions at recommended intervals based on pre-transfusion Hb, appropriate parental counselling and one-on-one communication. Previous studies have suggested that regular blood transfusions can impact their school attendance and that suboptimal blood transfusions may not alleviate anaemic symptoms, potentially disrupting a child's ability to concentrate in class.[11-13]

The type of chelation therapy significantly affected HRQOL, with Deferasirox users reporting superior physical, emotional, social functioning as well as total HRQOL scores compared to those using deferoxamine, deferiprone, or no chelation therapy ($p<0.05$ for most categories). This could be attributed to deferasirox which possesses more effective iron chelation properties, resulting in enhanced physical and emotional well-being. Also, its oral administration, which might be more convenient and better tolerable side effect profile compared to other chelators, resulted in better adherence and outcomes. The moderate negative correlation between HRQOL and the type of chelation therapy, which was statistically significant, highlights the importance of effective and patient-friendly chelation therapy in managing blood transfusion and improving HRQOL. To the best of our knowledge, there was no previously reported study regarding the correlation between the type of chelation therapy and HRQOL with TDTM children to compare our study observations with.

The findings of our study have several implications for clinical practice. Firstly, there is a need for targeted interventions to address the declining physical functioning and overall HRQOL in older children. Secondly, the significant impact of educational status on HRQOL highlights the importance of providing educational support and addressing the academic challenges faced by these children. Thirdly, age-specific interventions in the form of regular counselling for patients and parents at each visit should be an integral part of regular management. Additionally, optimizing chelation therapy is essential to enhance HRQOL in this population.

There were few limitations in our study. Firstly, the study's sample size was relatively small, which may limit the generalizability of the findings to a broader population of TDTM children. Larger studies with more diverse populations are required to confirm these results. Secondly, HRQOL was assessed by using the Peds QL questionnaire, which relies on self-reporting. Self-reported measures are prone to bias, including social desirability bias and recall bias, which may affect the accuracy of the responses. Thirdly, the study did not account for socioeconomic and environmental factors impacting HRQOL, such as family income, parental education, and living conditions. These variables could provide additional context for understanding the HRQOL outcomes.

## CONCLUSION

HRQOL was significantly affected among TDTM children. Physical functioning, school functioning, and overall HRQOL scores were decreased with age. The frequency of blood transfusions does not significantly impact overall HRQOL. Deferasirox was associated with higher HRQOL scores. Patients without chelation therapy exhibited the lowest HRQOL scores, underscoring the importance of effective chelation therapy in managing iron overload and enhancing HRQOL.

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