Impact of non-transfusion-dependent thalassemia on adult patients' health-related quality of life and work productivity in the Kingdom of Saudi Arabia and the United Arab Emirates

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BACKGROUND

- Thalassemia is a group of chronic, hereditary disorders associated with impaired red blood cell function and survival, caused by insufficient production of healthy hemoglobin (Hb)[1]
- Thalassemia is commonly categorized according to transfusion requirements: transfusion-dependent thalassemia (TDT) and non-transfusion-dependent thalassemia (NTDT)[1]
- While patients with NTDT do not require regular transfusions for survival, many experience substantial disease burden, and increased morbidity and mortality[2,3]
- Thalassemia is prevalent in the Middle East[4], but there is limited understanding of the patient-centric impacts of NTDT in the Kingdom of Saudi Arabia (KSA) and the United Arab Emirates (UAE)

OBJECTIVE

To investigate health-related quality of life and work productivity among patients with NTDT in the KSA and the UAE

METHODS

- Data were collected from the multi-national Adelphi Real World Thalassemia Disease Specific Programme™[5–8], a non-interventional cross-sectional survey of physicians and their patients from February to November 2024
- Patients provided informed consent and were aged ≥18 years, with a physician-confirmed diagnosis of α- or β-NTDT
  - Patients who had previously undergone gene therapy or hematopoietic stem cell transplantation or were involved in any clinical trial at the time of the survey were excluded
- Patients were invited to complete a patient self-completion form capturing demographics, patient-reported outcome measures (PROMs) (**Table 1**), impacts of NTDT, and patient treatment goals
- In the present analysis, data are reported from the KSA and the UAE and summarized descriptively; patient-reported outcome data were compared numerically with published population norms (PPNs) from the United States (US)
  - PPNs from the US were used because there are no Gulf Cooperation Council (GCC)-specific population norms available

Table 1. Patient-reported outcome measures included in this study

Functional Assessment of Chronic Illness Therapy (FACIT)-Fatigue scale, version 4[9]	• 13 items • Assesses fatigue and its impact on daily activities and function (7-day recall period) • Score range 0–52; higher scores indicate lower fatigue
Patient-Reported Outcomes Measurement Information System Physical Function (PROMIS PF), version 2 Short Form 8b[10]	• 8 items • Assesses patients' ability to carry out physical tasks and daily activities (no recall period) • T-score with range 0–100; higher scores indicate lower impairment
Work Productivity and Activity Impairment (WPAI)-Thalassemia[11]	• 6 items • Measures impairments in both paid work and daily activities due to thalassemia (7-day recall period) • Assesses absenteeism (work time missed), presenteeism (impairment while working), and overall work impairment in employed patients, and activity impairment in all patients • Score ranges 0–100; higher scores indicate greater impairment

FACIT, Functional Assessment of Chronic Illness Therapy; PROMIS PF, Patient-Reported Outcomes Measurement Information System Physical Function; WPAI, Work Productivity and Activity Impairment.

RESULTS

Patient characteristics

- This analysis included adult patients with NTDT from the KSA (N=15) and from the UAE (N=15)
  - 66.7% and 53.3% had β-NTDT, respectively
- Patient-reported characteristics are shown in **Table 2**
  - In patients from the KSA and the UAE, mean (standard deviation [SD]) age was 35.2 (6.2) and 34.0 (4.4) years, and most patients worked full or part time (80.0% for both countries)
  - Mean (SD) Hb levels over the previous 12 months[a] were 10.0 g/dL (1.4) and 10.1 g/dL (1.0) in the KSA and the UAE groups, respectively

[a]Physicians reported patients' typical (average) Hb level during the past 12 months, regardless of timing relative to transfusions.

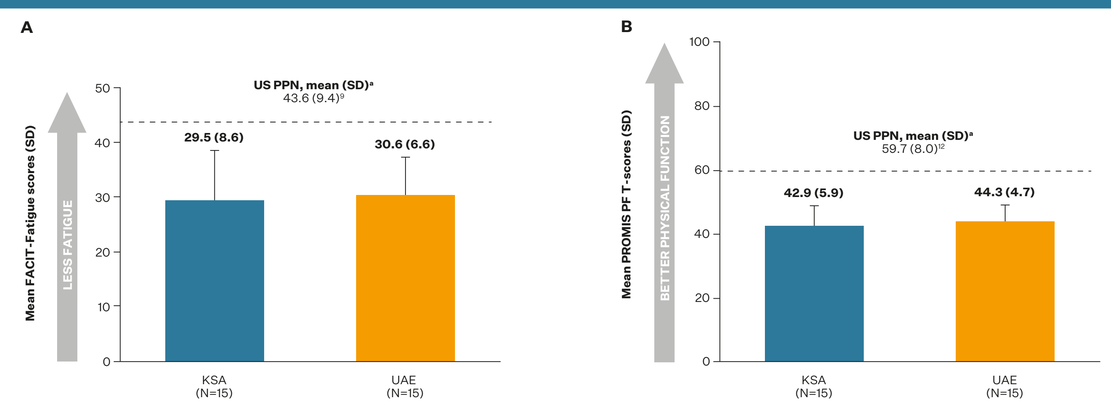
Table 2. Patient characteristics at the time of survey completion

	KSA N=15	UAE N=15
Age, years, mean (SD)	35.2 (6.2)	34.0 (4.4)
Female, n (%)	8 (53.3)	6 (40.0)
Thalassemia type, n (%)		
α-NTDT	5 (33.3)	7 (46.7)
β-NTDT	10 (66.7)	8 (53.3)
Current employment status, n (%)		
Working full time	6 (40.0)	11 (73.3)
Working part time	6 (40.0)	1 (6.7)
Homemaker	3 (20.0)	2 (13.3)
Unemployed	0 (0.0)	1 (6.7)
Average Hb levels in the past 12 months, g/dL[a], mean (SD)	10.0 (1.4)	10.1 (1.0)
Patients who had ever received a transfusion, n (%)[b]		
1–5 red blood cell transfusions	12 (80.0)	10 (66.7)
6–10 red blood cell transfusions	0 (0)	0 (0.0)
≥11 red blood cell transfusions	0 (0)	0 (0)
Patient has never received a red blood cell transfusion for their thalassemia	3 (20.0)	5 (33.3)

[a]Physicians reported patients' typical (average) Hb level during the past 12 months, regardless of timing relative to transfusions. [b]As reported by the patient's physician.
Hb, hemoglobin; KSA, Kingdom of Saudi Arabia; NTDT, non-transfusion-dependent thalassemia; SD, standard deviation; UAE, United Arab Emirates.

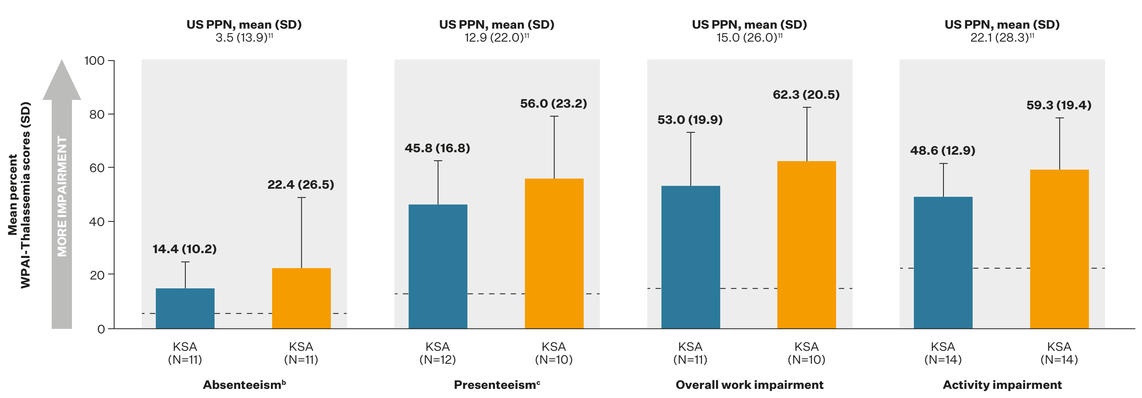
- FACIT-Fatigue scores (**Figure 1A**) and PROMIS PF T-scores (**Figure 1B**) were lower in patients with NTDT in the KSA and the UAE compared with population norms[9,12]
- WPAI-Thalassemia absenteeism, presenteeism, overall work impairment, and activity impairment scores were greater in patients with NTDT in the KSA and the UAE compared with population norms[11] (**Figure 2**)

Figure 1. FACIT-Fatigue and PROMIS PF T-scores in the KSA and the UAE



[a]Dashed lines represent the respective US PPN per category. US PPNs were used because there are no GCC-specific population norms available. FACIT, Functional Assessment of Chronic Illness Therapy; GCC, Gulf Cooperation Council; KSA, Kingdom of Saudi Arabia; NTDT, non-transfusion-dependent thalassemia; PPN, published population norm; PROMIS PF, Patient-Reported Outcomes Measurement Information System Physical Function; SD, standard deviation; UAE, United Arab Emirates; US, United States.

Figure 2. WPAI-Thalassemia in the KSA and the UAE[a]

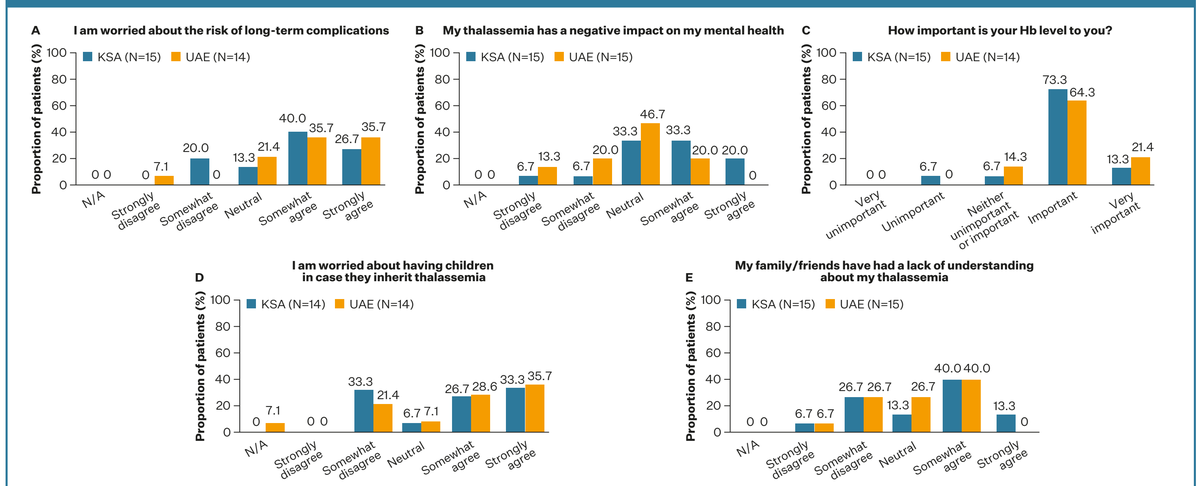


Absenteeism, presenteeism, and overall work impairment were assessed in employed patients; activity impairment was assessed in all patients. [a]Dashed lines represent the respective US PPN per category. US PPNs were used because there are no GCC-specific population norms available. [b]Absenteeism: work time missed. [c]Presenteeism: impairment while working. GCC, Gulf Cooperation Council; KSA, Kingdom of Saudi Arabia; NTDT, non-transfusion-dependent thalassemia; PPN, published population norm; PROM, patient-reported outcome measure; SD, standard deviation; UAE, United Arab Emirates; US, United States; WPAI, Work Productivity and Activity Impairment.

Patient-reported daily life impacts of NTDT

- Overall, 69.0% of patients agreed they had concerns regarding the risk of long-term complications associated with thalassemia (**Figure 3A**)
  - In addition, 36.7% of patients agreed thalassemia had a negative impact on their mental health (**Figure 3B**)
- The majority of patients (86.2%) felt that their Hb levels were important or very important (**Figure 3C**)
  - Concerns about passing along thalassemia to their children, and a lack of understanding of thalassemia among their family and friends, were also common (62.1% and 46.7%, respectively; **Figure 3D and 3E**)

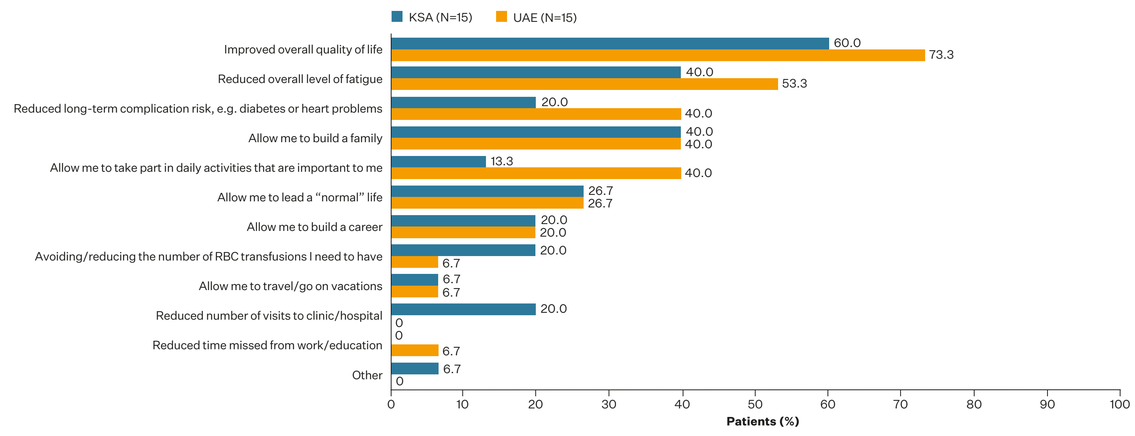
Figure 3. Patient-reported daily life impacts of NTDT[a]



[a]Base changes indicate instances where patients did not provide a response. Hb, hemoglobin; KSA, Kingdom of Saudi Arabia; N/A, not applicable; NTDT, non-transfusion-dependent thalassemia; UAE, United Arab Emirates.

- The most frequently reported important treatment goals desired by patients with NTDT were for an improvement in quality of life and a reduction in overall levels of fatigue (**Figure 4**)

Figure 4. Treatment goals reported as important by patients with NTDT



KSA, Kingdom of Saudi Arabia; NTDT, non-transfusion-dependent thalassemia; RBC, red blood cell; UAE, United Arab Emirates.

LIMITATIONS

- Participating patients in the KSA and the UAE may not reflect the general NTDT population
- Recall bias, a common limitation of surveys, might also have affected patient responses
  - However, PROMs were captured with short or no recall periods to minimize the possibility of recall bias
- US PPNs were used as a reference because there are no GCC-specific population norms available for FACIT-Fatigue, PROMIS PF, or WPAI
- The sample size was relatively small

CONCLUSIONS

- **Adult patients with α- or β-NTDT in the KSA and the UAE experience worse fatigue and greater impairment in physical function and work productivity relative to population norms**
- **Patients identified improvement in overall quality of life and reduction in fatigue as their most important goals when treating their NTDT**
- **This study highlights an unmet need for novel treatments to reduce the humanistic burden of NTDT in the KSA and the UAE**

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