

Impact of 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
- The symptoms of TDT, as well as the logistical burden of receiving regular transfusions, can result in significant impairments to quality of life and work productivity^{2,3}
 - The family and caregivers of patients with thalassemia can also experience substantial quality of life burden as part of the wider impacts of disease management^{2,4}
- Thalassemia is prevalent in the Middle East⁵, but there is limited understanding of the patient- and caregiver-centric impacts of TDT 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 TDT and caregivers of patients with TDT in the KSA and the UAE

METHODS

Data collection and analysis

- Data were collected from the multi-national Adelphi Real World Thalassemia Disease Specific Programme^{TM6–9}, a non-interventional cross-sectional survey of physicians and their patients from February to November 2024
- The present analysis included patients from the KSA and the UAE with a physician-confirmed diagnosis of α - or β -TDT, and caregivers of patients with α - or β -TDT (both aged ≥ 18 years)
 - Patients who had undergone gene therapy or hematopoietic stem cell transplantation or were involved in any clinical trial at the time of the survey were excluded

RESULTS

Patient characteristics

- This analysis included adult patients with TDT from the KSA (N=19) and from the UAE (N=23)
 - 73.7% and 78.3% had β -TDT, respectively
- Patient-reported characteristics are shown in **Table 2**
 - In patients from the KSA and the UAE, mean (standard deviation [SD]) age was 26.5 (3.6) and 29.4 (6.6) years, and 47.4% and 60.9%, respectively, worked full or part time
- Mean (SD) Hb levels over the previous 12 months^a were 8.5 (1.5) and 9.9 (0.6) g/dL in patients from the KSA and the UAE, respectively

^aPhysicians 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=19	UAE N=23
Age, years, mean (SD)	26.5 (3.6)	29.4 (6.6)
Female, n (%)	12 (63.2)	11 (47.8)
Thalassemia type, n (%)		
α -TDT	5 (26.3)	5 (21.7)
β -TDT	14 (73.7)	18 (78.3)
Current employment status, n (%)		
Working part time	8 (42.1)	4 (17.4)
Working full time	1 (5.3)	10 (43.5)
Student	3 (15.8)	5 (21.7)
Unemployed	4 (21.1)	3 (13.0)
Homemaker	3 (15.8)	1 (4.3)
Average Hb levels in the past 12 months, g/dL ^a , mean (SD)	8.5 (1.5)	9.9 (0.6)

^aPhysicians reported patients’ typical (average) Hb level during the past 12 months, regardless of timing relative to transfusions. Hb, hemoglobin; KSA, Kingdom of Saudi Arabia; SD, standard deviation; TDT, transfusion-dependent thalassemia; UAE, United Arab Emirates.

- This analysis included caregivers of patients with TDT from the KSA (N=28) and from the UAE (N=23) (**Table 3**)
 - Caregivers were predominantly parents (82.1% and 73.9%, respectively)^a

^aCaregiver-reported data do not correspond to patients reported in this analysis.

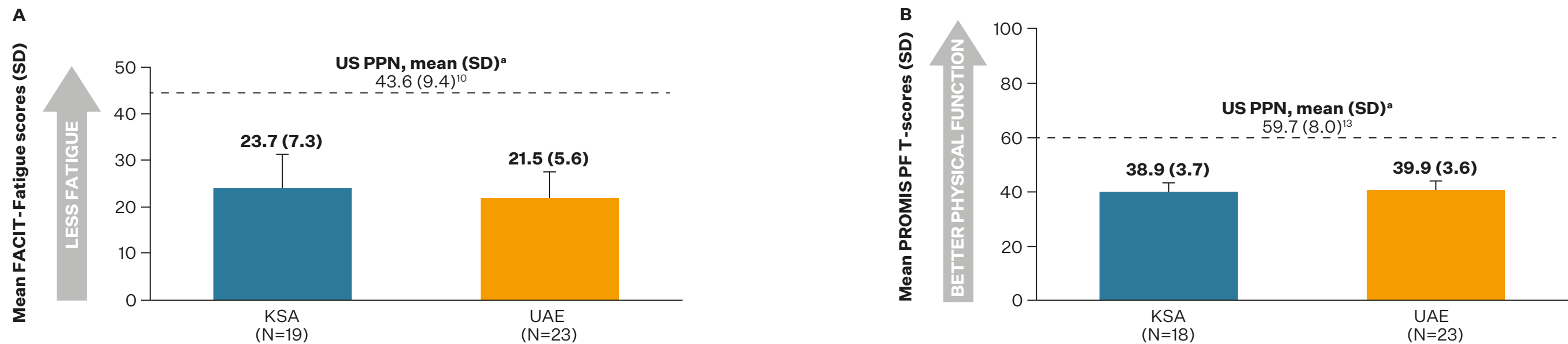
Table 3. Caregiver characteristics at the time of survey completion

	KSA N=28	UAE N=23
Age (years), mean (SD)	41.9 (5.9)	44.0 (7.6)
Female, n (%)	20 (71.4)	15 (65.2)
Caregiver relationship to patient with thalassemia, n (%)		
Parent	23 (82.1)	17 (73.9)
Partner/spouse	3 (10.7)	3 (13.0)
Another family member	2 (7.1)	2 (8.7)
Sibling	0 (0.0)	1 (4.3)
Current employment status, n (%) ^a		
Homemaker	17 (60.7)	14 (60.9)
Working full time	9 (32.1)	9 (39.1)
Working part time	2 (7.1)	0 (0.0)
On disability benefit	2 (7.1)	0 (0.0)

^aTwo caregivers reported themselves as both a ‘homemaker’ and ‘on disability benefit’ for current employment status. KSA, Kingdom of Saudi Arabia; SD, standard deviation; TDT, transfusion-dependent thalassemia; UAE, United Arab Emirates.

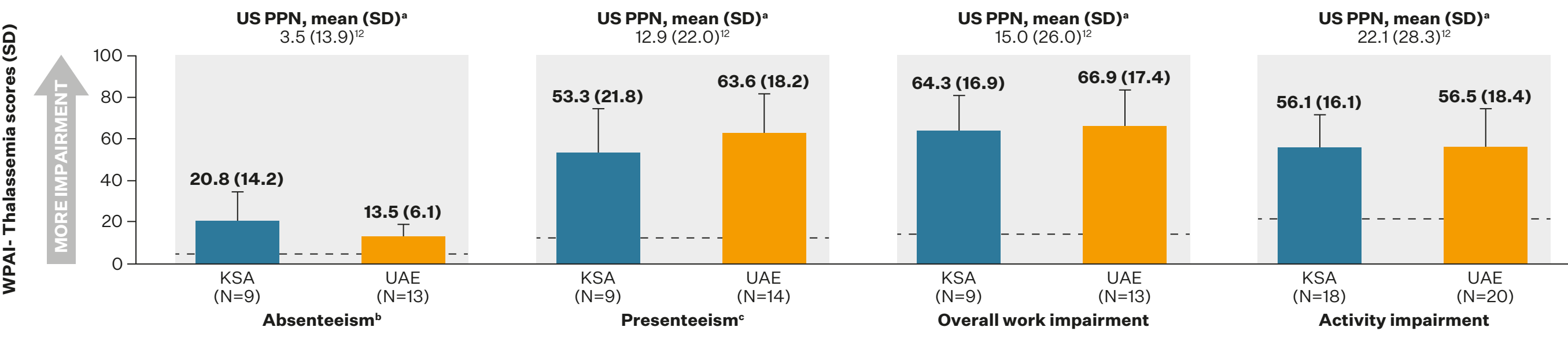
- FACIT-Fatigue scores (**Figure 1A**) and PROMIS PF T-scores (**Figure 1B**) were lower in patients with TDT in the KSA and the UAE compared with population norms^{10,13}
- WPAI-Thalassemia absenteeism, presenteeism, overall work impairment, and activity impairment were higher in patients with TDT in the KSA and the UAE compared with population norms¹² (**Figure 2**)

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



^aDashed 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; PPN, published population norm; PROMIS PF, Patient-Reported Outcomes Measurement Information System Physical Function; SD, standard deviation; TDT, transfusion-dependent thalassemia; UAE, United Arab Emirates; US, United States.

Figure 2. WPAI-Thalassemia in the KSA and the UAE



Absenteeism, presenteeism, and overall work impairment were assessed in employed patients; activity impairment was assessed in all patients. ^aDashed lines represent the respective US PPN per category. US PPNs were used because there are no GCC-specific population norms available. ^bAbsenteeism: work time missed. ^cPresenteeism: impairment while working. GCC, Gulf Cooperation Council; KSA, Kingdom of Saudi Arabia; PPN, published population norm; SD, standard deviation; TDT, transfusion-dependent thalassemia; UAE, United Arab Emirates; US, United States; WPAI, Work Productivity and Activity Impairment.

- Patient-reported impacts of TDT included (**Figure 3**):
 - Concerns regarding the risk of long-term complications associated with thalassemia (78.0% somewhat agree/strongly agree)
 - Negative impact on mental health (64.3% somewhat agree/strongly agree)
 - Difficulty managing transfusions around day-to-day activities (42.9% somewhat agree/strongly agree)

- Patients were invited to complete a patient self-completion form capturing demographics and patient-reported outcome measures (PROMs) (**Table 1**), and impacts of TDT
 - Caregivers (≥ 18 years) from the KSA and the UAE were invited to complete self-completion forms if accompanying patients to a physician consultation^a
- Data from patients and caregivers from the KSA and the UAE were summarized descriptively, and were compared numerically with published population norms (PPNs) from the United States (US)
 - US PPNs were used because there are no Gulf Cooperation Council (GCC)-specific population norms available

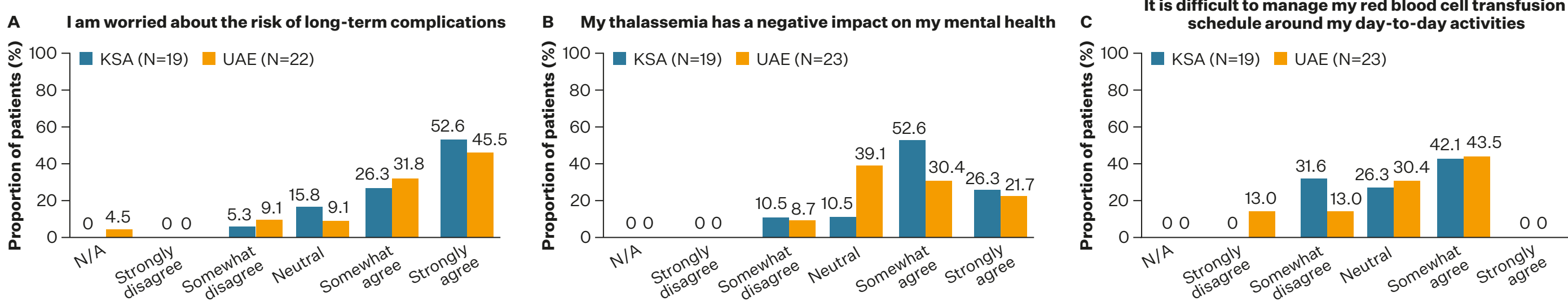
^aCaregiver-reported data do not correspond to patients reported in this analysis.

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

Functional Assessment of Chronic Illness Therapy (FACIT) -Fatigue scale, version 4¹⁰	<ul style="list-style-type: none">13 itemsAssesses 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 8b¹¹	<ul style="list-style-type: none">8 itemsAssesses 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) -Thalassemia¹²	<ul style="list-style-type: none">6 itemsMeasures 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 patientsScore 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.

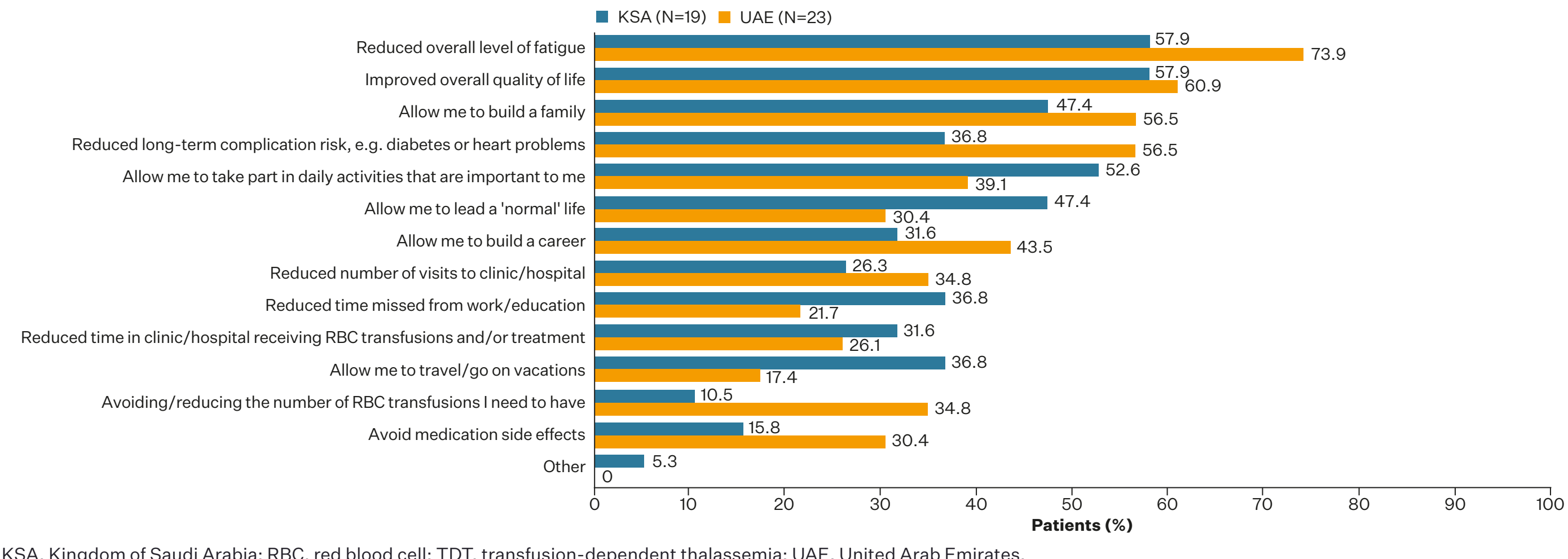
Figure 3. Patient-reported daily life impacts of TDT^a



^aBase changes indicate instances where patients did not provide a response. KSA, Kingdom of Saudi Arabia; N/A, not applicable; TDT, transfusion-dependent thalassemia; UAE, United Arab Emirates.

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

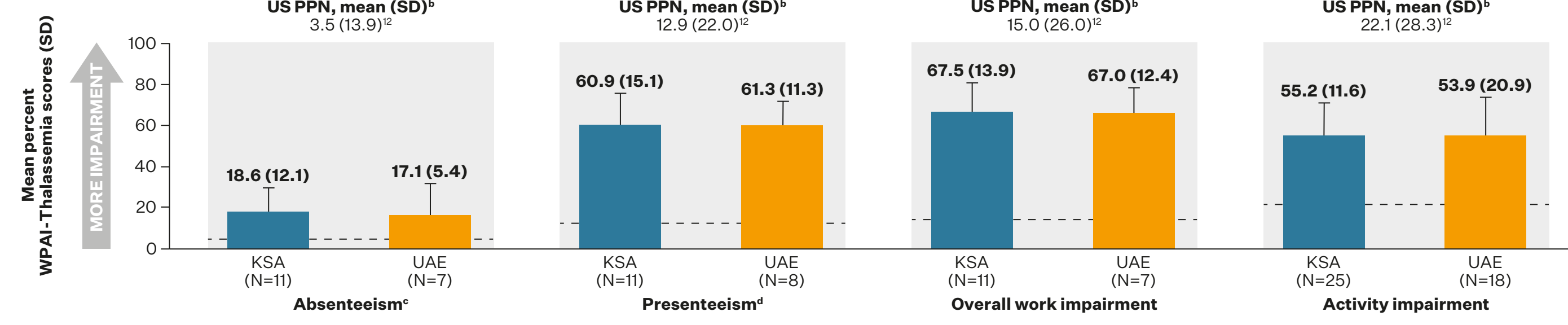
Figure 4. Treatment goals reported as important by patients with TDT



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

- WPAI-Thalassemia absenteeism, presenteeism, overall work impairment, and activity impairment scores were higher in caregivers of patients with TDT in the KSA and the UAE compared with population norms¹²

Figure 5. Caregiver-reported WPAI-Thalassemia^a



^aCaregiver-reported data do not correspond to patients reported in this analysis. Absenteeism, presenteeism, and overall work impairment were assessed in employed caregivers; activity impairment was assessed in all caregivers. ^bDashed lines represent the respective US PPN per category. US PPNs were used because there are no GCC-specific population norms available. ^cAbsenteeism: work time missed. ^dPresenteeism: impairment while working. GCC, Gulf Cooperation Council; KSA, Kingdom of Saudi Arabia; PPN, published population norm; SD, standard deviation; TDT, transfusion-dependent thalassemia; UAE, United Arab Emirates; US, United States; WPAI, Work Productivity and Activity Impairment.

LIMITATIONS

- Participating patients and caregivers in the KSA and the UAE may not reflect the general TDT/caregiver population
- Recall bias, a common limitation of surveys, might have affected responses
 - However, patient- and caregiver-reported outcome tools 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 -TDT in the KSA and the UAE experience worse fatigue and greater impairment in physical function and work productivity relative to population norms
- Caregivers of patients with α - or β -TDT in the KSA and the UAE also experienced impaired work productivity
- This study highlights an unmet need to reduce the humanistic burden of TDT in the KSA and the UAE

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