Clinical burden and transfusion-related outcomes in transfusion-dependent thalassemia in the Kingdom of Saudi Arabia and the United Arab Emirates

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BACKGROUND

- Thalassemia is a group of chronic, inherited blood disorders resulting from an underproduction of healthy hemoglobin (Hb)[1]
- Thalassemia is associated with ineffective erythropoiesis and hemolysis, leading to chronic anemia and serious complications that can lead to hospitalization, significantly impair patients' health-related quality of life, and contribute to the clinical burden of thalassemia[2–4]
- Thalassemia can be categorized based on transfusion requirements[5,6]
  - Transfusion-dependent thalassemia (TDT) necessitates lifelong regular transfusions for survival, imposing a substantial burden on patients and their families[1,6–8]
  - Additionally, regular transfusions can be associated with serious complications, including infection, alloimmunization, and iron overload requiring chelation therapy, which further increase disease burden[7–11]

OBJECTIVE

The aim of this study was to characterize the clinical burden and transfusion-related outcomes of patients with TDT in the Kingdom of Saudi Arabia (KSA) and the United Arab Emirates (UAE)

METHODS

- Data were drawn from the Adelphi Real World Thalassemia Disease Specific Programme™ [12–15], a cross-sectional, retrospective survey of physicians and their adult patients with a physician-confirmed diagnosis of TDT (α- or β-thalassemia), conducted from February to November 2024
- Patients participating in any clinical trial at the time of the survey, and those with a history of gene therapy or hematopoietic stem cell transplantation, were excluded
- Physicians reported patient demographics and clinical characteristics in a patient record form (PRF) for up to 15 consecutive patient consultations
  - Physician-reported data included concomitant complications (complications associated with thalassemia) ever experienced, current and historical transfusion-related outcomes (including complications and hospitalizations), and current treatment patterns
- Each patient for whom the physician completed a PRF was invited to complete a voluntary patient self-completion form (PSC)
  - Patient-reported data included time spent receiving transfusions and traveling to and from the hospital, how bothered they were by the time taken to receive transfusions, and how anxious they felt when receiving transfusions
- Data are reported from the KSA and the UAE, and are summarized descriptively

RESULTS

Patient characteristics

- PRF data were collected from 122 adult patients with TDT, 42 of whom completed a PSC; patient characteristics are presented in **Table 1**

Table 1. Patient characteristics[a]

	KSA N=60	UAE N=62
Age, years, mean (SD)	24.8 (5.7)	27.1 (6.5)
Female, n (%)	36 (60.0)	31 (50.0)
Thalassemia genotype, n (%)		
α-thalassemia	17 (28.3)	16 (25.8)
β-thalassemia	43 (71.7)	46 (74.2)
Genetic test to diagnose thalassemia genotype, n (%)		
Yes	56 (93.3)	61 (98.4)
No	4 (6.7)	1 (1.6)
Average Hb levels in the past 12 months, g/dL, mean (SD)[b]	8.8 (1.3)	9.8 (0.9)

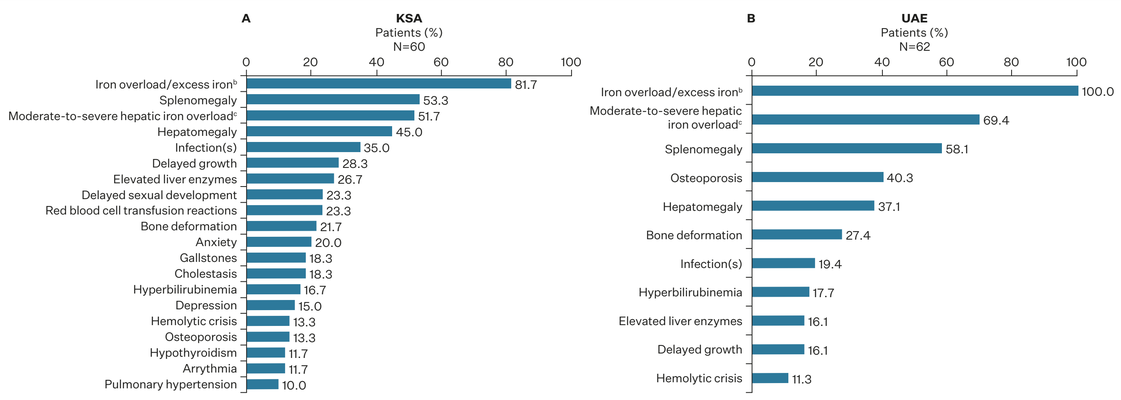
[a]Data shown are physician-reported characteristics at the time of completing the PRF. PRF data were collected from 122 adult patients with TDT. [b]Physicians reported patients' typical (average) haemoglobin level during the past 12 months, regardless of timing relative to transfusions. Hb, hemoglobin; KSA, Kingdom of Saudi Arabia; PRF, patient record form; SD, standard deviation; TDT, transfusion-dependent thalassemia; UAE, United Arab Emirates.

Most frequent complications associated with thalassemia ever experienced[a]

- Patients with TDT experienced a broad range of complications; the most frequent complications associated with thalassemia ever experienced were:
  - In the KSA: iron overload (81.7%), splenomegaly (53.3%), and moderate-to-severe hepatic iron overload (51.7%) (**Figure 1A**)
  - In the UAE: iron overload (100.0%), moderate-to-severe hepatic iron overload (69.4%), and splenomegaly (58.1%) (**Figure 1B**)
  - Complications ever experienced by <10% of patients are presented in **Supplemental Figure 1**

[a]Data shown are physician-reported characteristics at the time of completing the PRF. PRF data were collected from 122 adult patients with TDT.

Figure 1. Complications associated with thalassemia ever experienced by ≥10% of patients[a]



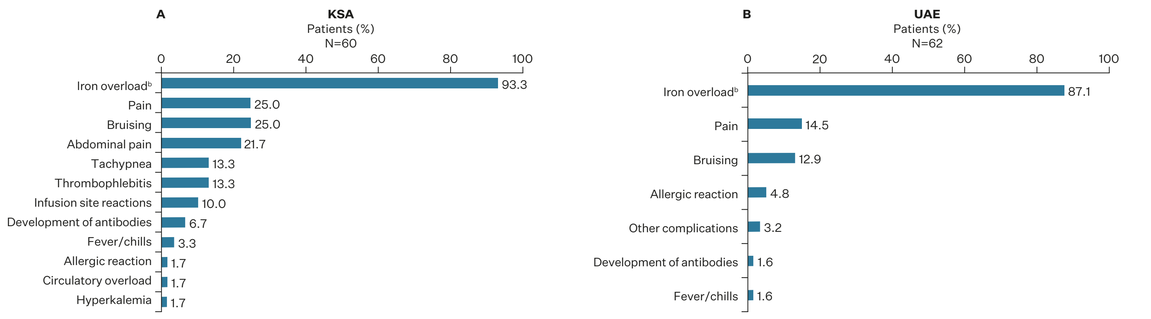
[a]Data shown are physician-reported characteristics at the time of completing the PRF. PRF data were collected from 122 adult patients with TDT. [b]Iron overload defined per physician's assessment. [c]Moderate-to-severe hepatic iron overload defined as >7.0 mg/g or >57 µmol/g. KSA, Kingdom of Saudi Arabia; PRF, patient record form; TDT, transfusion-dependent thalassemia; UAE, United Arab Emirates.

Complications and treatment burden due to transfusions

- Overall, 100.0% and 90.3% of patients with TDT in the KSA and the UAE, respectively, experienced acute or long-term complications due to transfusions
  - The most frequent in both the KSA and the UAE were iron overload, pain, and bruising (**Figure 2**)[a]
- In both the KSA and the UAE, patients reported spending an average of 3 hours at hospital receiving their transfusions, and traveling for an average of 0.6 hours each way for their appointment[b]
  - Of patients who completed a PSC, 73.6% (KSA) and 73.9% (UAE) reported feeling somewhat or moderately bothered by the time taken to receive transfusions, with 4.3% in the UAE very bothered (N=42) (**Supplemental Figure 2A**)
  - Of patients who completed a PSC, 63.2% (KSA) and 63.6% (UAE) reported feeling somewhat or moderately anxious when receiving transfusions, with 10.5% (KSA) and 9.1% (UAE) feeling very anxious (N=41)[c] (**Supplemental Figure 2B**)
- Blood supply issues in the 12 months prior to survey completion affected 66.7% of patients in the KSA (N=54) and 54.8% in the UAE (N=62)
  - The most frequent blood supply issues in both the KSA and the UAE were low stocks of blood type to match the patient, reliance on family or friends for blood, or donor blood was poor quality (**Supplemental Table 1**)[d]

[a]Data shown are physician-reported characteristics at the time of completing the PRF. PRF data were collected from 122 adult patients with TDT. [b]PSC data were collected from 42 adult patients with TDT. [c]Based on N=41; 1 patient was not included in the base due to providing more than one answer. [d]Based on N=116; 6 patients were not included in the base due to physician response of 'don't know'.

Figure 2. Complications experienced due to transfusions[a]



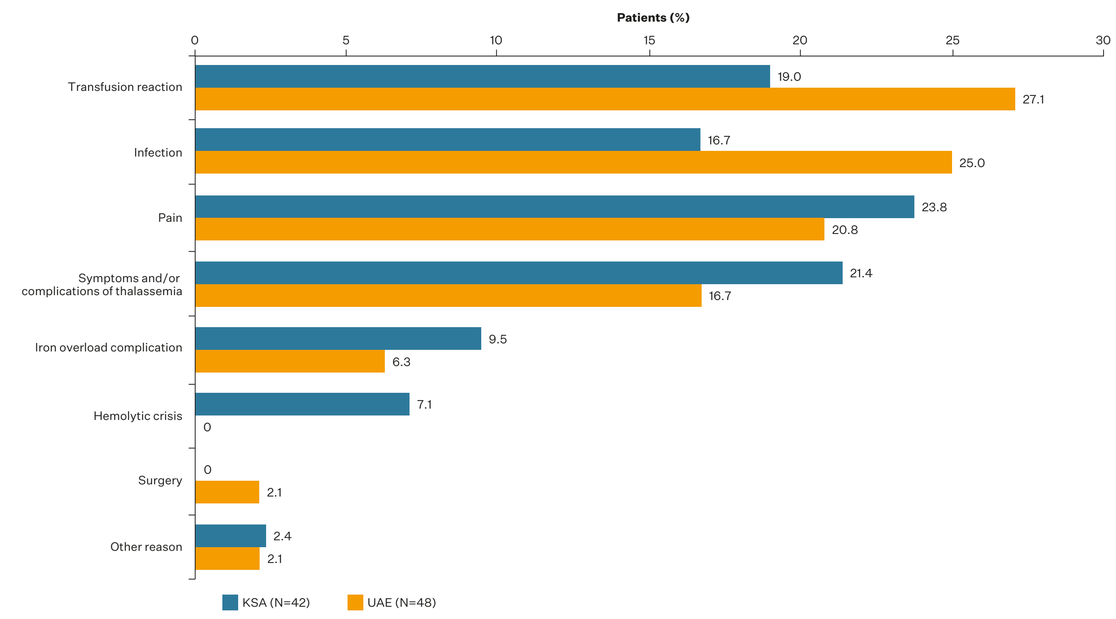
[a]Data shown are physician-reported characteristics at the time of completing the PRF. PRF data were collected from 122 adult patients with TDT. [b]Iron overload defined per physician's assessment. KSA, Kingdom of Saudi Arabia; PRF, patient record form; TDT, transfusion-dependent thalassemia; UAE, United Arab Emirates.

Thalassemia-related complications that led to hospitalization[a]

- In the KSA (N=42/60) and the UAE (N=48/62), respectively, 70.0% and 77.4% of patients experienced hospitalization in the past 12 months due to thalassemia-related complications (**Figure 3**)

[a]Data shown are physician-reported characteristics at the time of completing the PRF. PRF data were collected from 122 adult patients with TDT.

Figure 3. Reasons for most recent hospitalization in the past 12 months[a]



[a]Data shown are physician-reported characteristics at the time of completing the PRF. PRF data were collected from 122 adult patients with TDT, of whom 90 were hospitalized in the past 12 months. KSA, Kingdom of Saudi Arabia; PRF, patient record form; TDT, transfusion-dependent thalassemia; UAE, United Arab Emirates.

Treatment patterns[a]

- Most patients in the KSA and the UAE were prescribed treatments for their thalassemia or related complications, including chelation therapies (deferoxamine, deferiprone, deferasirox) (95.0% of patients in the KSA; 100.0% in the UAE), folic acid (86.7% in the KSA; 93.5% in the UAE), and calcium (63.3% in the KSA; 50.0% in the UAE)
  - Data for treatments prescribed to ≥10% of patients in either the KSA or the UAE are presented in **Table 2**; data for treatments prescribed to <10% of patients are presented in **Supplemental Table 2**

[a]Data shown are physician-reported characteristics at the time of completing the PRF. PRF data were collected from 122 adult patients with TDT.

Table 2. Most frequent treatments currently prescribed for thalassemia or related complications[a,b]

Which treatments are the patient prescribed for their thalassemia at time of survey completion? n (%)	KSA N=60	UAE N=62
Chelation therapies combined	57 (95.0)	62 (100.0)
Deferasirox	28 (46.7)	28 (45.2)
Deferoxamine	26 (43.3)	16 (25.8)
Deferiprone	8 (13.3)	19 (30.6)
Folic acid	52 (86.7)	58 (93.5)
Calcium	38 (63.3)	31 (50.0)
Vitamin D	27 (45.0)	23 (37.1)
Calcifediol / 25-OHD	8 (13.3)	14 (22.6)
Thyroid hormone replacement	7 (11.7)	4 (6.5)

[a]Data presented for treatments prescribed to ≥10% of patients in either KSA or UAE only. [b]Data shown are physician-reported characteristics at the time of completing the PRF. PRF data were collected from 122 adult patients with TDT. 25-OHD, 25-hydroxy vitamin D; KSA, Kingdom of Saudi Arabia; PRF, patient record form; TDT, transfusion-dependent thalassemia; UAE, United Arab Emirates.

LIMITATIONS

- Participating patients may not be representative of the general TDT population
- Recall bias, a common limitation of surveys, might have affected physician and patient responses
  - Physicians had the ability to refer to patients' records while completing the PRF, minimizing the possibility of recall bias

CONCLUSIONS

- **In both the KSA and the UAE, adult patients with TDT experience considerable clinical challenges and substantial transfusion-related burden**
  - **The most common complications experienced in both regions were iron overload, splenomegaly, and moderate-to-severe hepatic iron overload**
  - **Up to 100% of patients with TDT in the KSA and 90.3% in the UAE experienced acute or long-term complications due to transfusions**
  - **Blood supply issues in the 12 months prior to survey completion affected 66.7% of patients in the KSA and 54.8% in the UAE**
- **These data highlight an unmet need for novel therapies to reduce disease complications, transfusion requirements, and treatment burden**

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All data are the intellectual property of Adelphi Real World; Agios Pharmaceuticals, Inc. subscribed to this survey and did not influence the original survey through either contribution to the design of questionnaires or data collection.

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Supplemental data are available via the QR code

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