Understanding health literacy among patients with thalassemia: Results from a global patient survey by the Thalassemia Advocacy Advisory Council

P6421

Sujit Sheth, MD[1], Lily Cannon[2], Laurice Levine[3], Ralph Colasanti[4], Jill Deitrick[5], Tobias Larkin[6], Craig Butler[4], Dina Steagall[7,8], Eleni Michalaki[9], Holly John[10], Keely S. Gilroy, PhD[10], Maria Domenica Cappellini, MD[11]

[1]Division of Hematology and Oncology, Department of Pediatrics, Weill Cornell Medicine, New York, NY, USA; [2]Thalassaemia International Federation, Nicosia, Cyprus; [3]Independent contributor, Petaluma, CA, USA; [4]Cooley's Anemia Foundation, New York, NY, USA; [5]Individual contributor, Sayre, PA, USA; [6]Individual contributor, Taoyuan, Taiwan; [7]Abrasta (Brazilian Association of Thalassemia), São Paulo, Brazil; [8]ABRALE (Brazilian Blood Cancer Organization), São Paulo, Brazil; [9]Individual contributor, Thessaloniki, Greece; [10]Agios Pharmaceuticals, Inc., Cambridge, MA, USA; [11]Fondazione IRCCS Ca' Granda Ospedale Maggiore Policlinico, SC Medicina ad Indirizzo Metabolico, Milan, Italy

BACKGROUND

- Thalassemia is a rare, under-recognized, hereditary hemolytic anemia with a variable clinical presentation, resulting in a variety of symptoms and complications that can substantially impact patient health-related quality of life[1–4]
  - Thalassemia has historically been most prevalent in the Middle East, the Mediterranean basin, and Southeast Asia; however, due to recent migration, it has become more common worldwide, including in Northern Europe and the United States[5,6]
  - Despite advances in the management of thalassemia, significant unmet needs remain in this patient population[7,8]
- The Thalassemia Advocacy Advisory Council (AAC), an international multistakeholder group of patients, caregivers, advocacy organizations, and healthcare professionals (HCPs) (**Supplemental Figure 1**), was formed as a novel approach to better understand the unmet needs of the thalassemia community, and to support initiatives to improve patient outcomes and care[9–11]
  - An assessment and subsequent evidence audit of published literature and advocacy group/community-based research identified health literacy as a critical gap for patients[9,10]
  - Based on these findings, the Thalassemia AAC developed a patient survey to gain insights into the global community's perspectives and to identify strategies to potentially address health literacy needs and support informed patient advocacy

OBJECTIVE

To report the quantitative results from the Thalassemia AAC global patient survey

METHODS

Survey and participation criteria

- A bespoke, self-administered, approximately 12–15-minute survey consisting of 6 sections was developed by the Thalassemia AAC (**Figure 1**)[12]
- Participants included adults (aged ≥18 years) with α- or β-thalassemia, excluding those with α- or β-thalassemia trait or patients currently enrolled in a clinical trial involving mitapivat (e.g. ENERGIZE [NCT04770753], ENERGIZE-T [NCT04770779], or AG348-C-010 [NCT03692052])
- All participants provided informed consent prior to taking part in the survey, and had the right to end their participation at any time

Figure 1. Thalassemia AAC survey sections[12]

1. Screening	• To gather information and assess survey eligibility
2. Demographics	• Optional questions to better understand the patient profile
3. Channels and formats	• To understand where patients search for health information and their preferred content format
4. Disease knowledge	• To collect information on disease awareness and understanding
5. Challenges and barriers	• To understand what aspects of life, such as work responsibilities, healthcare system issues, or family dynamics, make it difficult to effectively manage their thalassemia
6. Motivators	• To describe factors that may influence the patient to seek an increased understanding and self-management of disease

AAC, Advocacy Advisory Council.

Survey recruitment and data collection

- Participants were invited to take part via email and were recruited via a specialist survey recruitment agency or a patient advocacy organization (PAO) network:
  - Panel-based recruitment and data collection were conducted by Vitreous World between March 15 and April 17, 2024
  - PAO network recruitment was conducted by the Thalassaemia International Federation between August 1 and October 31, 2024
- Participants completed the survey online and data were captured electronically
  - Respondents answered single-choice, multiple-choice, scaled, and free-text questions
  - The data captured were aggregated into Excel data tables
- Survey responses were summarized descriptively as number and percentage for categorical variables, and net percentages for continuous variables

RESULTS

Demographic and disease characteristics

- 122 patients with thalassemia from the United States, Brazil, Italy, Greece, the United Arab Emirates, Saudi Arabia, and Kuwait participated in the survey
- Demographic and disease characteristics are reported in **Table 1**
  - The majority of patients (64%, n=78) were aged 25–44 years and had been diagnosed with β-thalassemia (77%, n=94)
  - Most patients (77%, n=94) report consulting a hematologist for their thalassemia treatment, with 27% (n=32) reporting monthly appointments and 20% (n=24) a bi-weekly visit schedule

Table 1. Participant demographic and disease characteristics

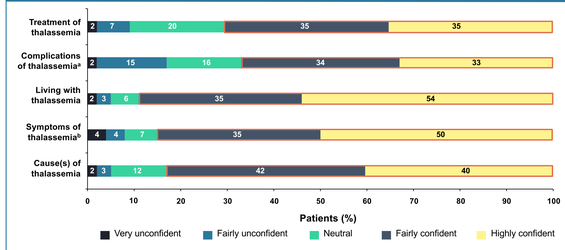
Demographic and disease characteristics, n (%)	Patients (N=122)
Location	
Brazil	25 (20)
Greece	15 (12)
Italy	25 (20)
Kuwait	7 (6)
Saudi Arabia	10 (8)
United Arab Emirates	15 (12)
United States	25 (20)
Age, years, n (%) [mean]	
18–24	9 (7) [21.1]
25–34	40 (33) [30.0]
35–44	38 (31) [38.6]
45–54	23 (19) [48.9]
55–64	7 (6) [62.7][a]
≥65	5 (4) [62.7][a]
Sex[b]	
Female	71 (58)
Male	50 (41)
Highest degree of education completed[b]	
Some high school	3 (2)
High school	30 (25)
Bachelor's degree	56 (46)
Master's degree	23 (19)
PhD or higher	7 (6)
Trade school	2 (2)
Thalassemia diagnosis	
α-thalassemia[c]	28 (23)
β-thalassemia[d]	90 (74)
α- and β-thalassemia[e]	4 (3)
Type of HCP most regularly consulted for thalassemia treatment	
Primary care physician	14 (11)
Nurse practitioner/physician assistant/allied health professional	10 (8)
Hematologist/oncologist	94 (77)
Other	2 (2)
Does not see an HCP about their thalassemia	2 (2)
Frequency of thalassemia-treating HCP consultations	
Weekly	6 (5)
Bi-weekly	24 (20)
Once every 3 weeks	11 (9)
Monthly	32 (27)
Bi-monthly	13 (11)
Quarterly	18 (15)
Twice a year	8 (7)
Once a year	3 (3)
Only occasionally	5 (4)

[a]Mean for age 55+ was 62.7 years. [b]One participant selected "Prefer not to say" as a response to this question; percents for sex and highest degree of education completed are based on the total respondents that answered the question (N=122)
[c]α-thalassemia included α-thalassemia major, hemoglobin (Hb) Barts, α-thalassemia intermedia, and HbH disease, and excluded α-thalassemia trait, minor, or carrier; [d]β-thalassemia included β-thalassemia major, β-thalassemia intermedia, and HbE/β-thalassemia, and excluded β-thalassemia trait, minor, carrier, or sickle β-thalassemia; [e]Four participants selected that they were diagnosed with both α- and β-thalassemia.
Hb, hemoglobin; HbE, hemoglobin E; HbH, hemoglobin H; HCP, healthcare professional.

Thalassemia disease knowledge

- Patients reported a high confidence in their knowledge of thalassemia, with the majority stating that they were confident regarding their understanding of the symptoms of thalassemia (85%, n=104) and living with the disease (89%, n=109) (**Figure 2**)
- The majority of patients (96%, n=118) also reported that their knowledge of thalassemia had improved to some extent over the previous 5 years (**Supplemental Figure 2**)
  - 43% (n=53) indicated that their knowledge of thalassemia had "improved a lot" during this time

Figure 2. Patient-reported confidence in their knowledge of thalassemia aspects (N=122)

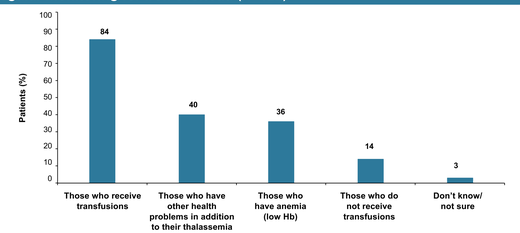


[a]Complications were defined as involving a worsening in the severity of the disease or the development of new signs, symptoms, or pathologic changes, i.e. infections or heart disease.
[b]A symptom was defined as a physical or mental manifestation of thalassemia apparent to the patient, i.e. fatigue or pale skin.

Thalassemia disease knowledge (cont.)

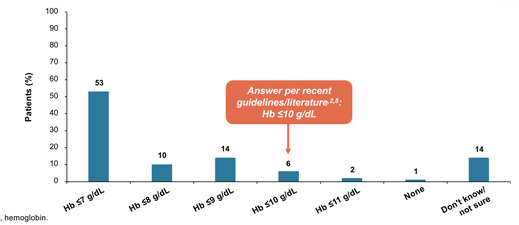
- Almost all patients (98%, n=119) recognized that if not treated properly, thalassemia can lead to serious complications that may require future treatment
- However, only a small minority of patients (14%, n=17) were aware that patients with non-transfusion-dependent thalassemia require monitoring for iron overload (**Figure 3**)
- Even fewer patients (6%, n=7) correctly recognized that a hemoglobin (Hb) level of ≤10 g/dL is associated with an increased complication risk; more than half (53%, n=65) incorrectly selected a threshold of ≤7 g/dL (**Figure 4**)

Figure 3. Participant beliefs of which patients with thalassemia should receive regular monitoring for iron overload (N=122)



Note: This was a multiple-choice question, participants had the opportunity to select more than one answer and were not limited to select the one response for the group they thought needed monitoring the most. Hb, hemoglobin.

Figure 4. Patient beliefs of the Hb level associated with thalassemia complications (N=122)

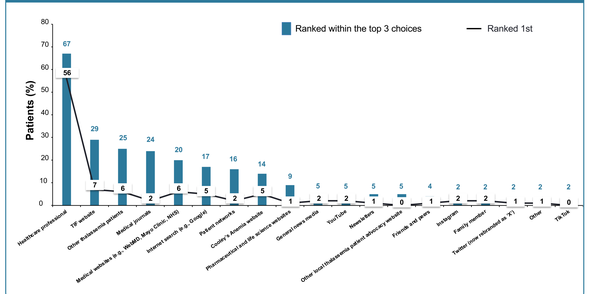


Hb, hemoglobin.

Thalassemia information sources utilized by patients

- Patients reported using a variety of information sources, including internet search engines, social media, and peers (**Supplemental Figure 3**)
- However, HCPs were the most frequently reported source of thalassemia management information, with two-thirds (67%, n=82) of patients identifying them as their most trusted source (**Figure 5**)

Figure 5. Trusted thalassemia information sources reported by patients (N=122)



NHS, National Health Service; TIF, Thalassaemia International Federation.

CONCLUSIONS

- **Despite patients reporting high confidence in disease understanding, several key knowledge gaps were identified**
  - **Of particular importance, most patients were unaware of the Hb levels associated with increased rates of complications, and the need for regular monitoring of patients with non-transfusion-dependent thalassemia**
  - **The responses regarding Hb may reflect the lower treatment targets still used by many providers, and therefore a lack of awareness among HCPs**
- **HCPs were the most frequently used source of information for patients, and the most trusted**

Overall, these findings suggest a potential overestimation of disease understanding among respondents

Efforts to support clinicians with patient education may provide an opportunity to help address these knowledge gaps

LIMITATIONS

- A limitation of this survey is that its questions are bespoke and have not been validated
- Additionally, convenience sampling was used, as recruitment involved targeting patients who were engaged with PAOs and may have been more educated about their disease; therefore, the responses may not be representative of all patients with thalassemia, potentially leading to an underestimation of health literacy needs

This study was funded by Agios Pharmaceuticals, Inc.

Acknowledgments: Thank you to the patients, caregivers, advocates, and healthcare professionals of the Thalassemia AAC for their valuable contributions to this study. Medical writing assistance was provided by Nia Higgs, MPharm, of Adelphi Group, Macclesfield, UK, funded by Agios Pharmaceuticals, Inc.

References: 1. Taher AT et al. N Engl J Med 2021;384:727–43; 2. Musallam KM et al. Hemasphere 2022;6:2103–04; 3. Taher AT et al. Lancet 2018;391:155–67; 4. Musallam KM et al. Poster presentation at the European Hematology Association (EHA) Congress 2025, Milan; PF1192; 5. Kattamis A et al. Eur J Haematol 2020;105:692–703; 6. Musallam KM et al. Am J Hematol 2023;98:1436–51; 7. Musallam KM et al. eJHaem 2024;5:541–47; 8. Taher AT et al. Guidelines for the management of non–transfusion-dependent β-thalassemia (3rd ed.). Thalassaemia International Federation; 2023. Accessed Aug 2025. 9. Cannon L et al. 16th International Conference on Thalassemia and Haemoglobinopathies and the 18th TIF International Conference for Patients and Parents 2023; 10. Sheth S et al. Blood 2023;142(Suppl 1):5251; 11. Sheth S et al. Blood 2024;144(Suppl 1):7949. AAC, Advocacy Advisory Council. 12. Cappellini MD et al. European Hematology Association (EHA) Library. 2025; PB3545.

Disclosures: SS: Agios Pharmaceuticals, Inc., Bristol Myers Squibb (Celgene), and Novo Nordisk – research funding; Agios Pharmaceuticals, Inc., BEAM, Bristol Myers Squibb (Celgene), Chiesi, and Novo Nordisk – consultancy/advisory board fees; CCO, PER, Plexus – honoraria; CRISPR Therapeutics/Vertex – participation in a steering committee. **LC:** Agios Pharmaceuticals, Inc. – honoraria provided to the Thalassaemia International Federation. **LL:** Agios Pharmaceuticals, Inc. – honoraria; Vertex – consultancy. **RC, JD, TL, CB, DS, and EM:** Agios Pharmaceuticals, Inc. – honoraria. **HJ and KSG:** Agios Pharmaceuticals, Inc. – employee and shareholder. **MDC:** Agios Pharmaceuticals, Inc., Bristol Myers Squibb (Celgene), Novo Nordisk, Pharmacosmos, Sanofi-Genzyme, Silence, Vifor – consultancy.

References and supplemental materials are available via the QR code

This study was funded by Agios Pharmaceuticals, Inc.
Presented at the 67th American Society of Hematology (ASH) Annual Meeting and Exposition, December 6–9, 2025, Orlando, FL, USA, and virtual