Thalassemia

Enhancing Patients' Disease Understanding for Improved Outcomes

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Contents

Prologue	01
Executive Summary	05
AAC Members	08
Introduction	09
Survey Process	13
Who Answered the Survey	15
Findings	17
Conclusions	30
Calls to Action	32
References	33
Acknowledgments	35

Prologue
Hematologist View

Thalassemia is an inherited blood disease and is a lifelong condition. A lack of disease understanding among many clinical experts contributes to widespread challenges in its effective management. There are evidence-based guidelines to support the appropriate management of thalassemia. Determining the best way to share this knowledge with those who need it is paramount.

Dr. Sujit Sheth

Dr. Maria Domenica Cappellini

Through partnering with our patients and the broader thalassemia community we have dedicated our careers to understanding and advancing thalassemia care. Despite major advances in the understanding and management of thalassemia over the last several decades, as specialists in the field, we see several remaining challenges in helping these patients. Around the world, the management of thalassemia, a benign hematological disease, varies significantly, few patients are seen in specialist clinics, and the majority are cared for by hematologists who focus on cancer. Physicians must also face misconceptions about the disease, overcome regional differences in resource availability, and navigate cultural sensitivities that may negatively impact patient care. There's so much to consider for these patients, but there is also so much at stake, and we must do our best on behalf of those we serve.

Health literacy, which includes knowledge about the disease, how and why it might affect someone and what complications may arise is extremely important. Unfortunately, as physicians we can't be there for patients all the time, and collaboration in the management of this chronic disease is critical for the health and wellbeing of our patients. We must ensure patients understand why it's so important for them to actively manage their thalassemia effectively.

Hematologist View Continued

In partnership with the Thalassemia Advocacy Advisory Council (AAC), which brings together a diverse range of voices from the global thalassemia community, we developed a survey to better understand health literacy needs of patients, with the goal of identifying opportunities for improvements in patient care. The survey collected information from 122 people across seven countries, giving us valuable insight into the lives of adults with thalassemia. This white paper summarizes their lived experiences, outlining key needs and actionable steps that we as partners can take to bring about changes that improve thalassemia care and outcomes.

The results of our patient survey indicate that healthcare professionals (HCPs) are the most trusted source of disease-related information, but HCPs may have limited opportunities for hands-on experience in the management of this underrecognized disease, resulting in knowledge gaps. We must be able to answer any question, confidently engage and guide our patients, and utilize the thalassemia clinical guidelines that have been thoughtfully developed by experts to inform treatment decisions. Thalassemia was a predominantly pediatric disease until a few decades ago, but now the majority of patients in the developed world are adults. Adult hematologists may have little experience with the disease, and patients still rely on pediatric hematologists in many settings. To help address the gaps in patients' understanding of the disease, as revealed by the survey, hematologists and generalists may need a more complete understanding of thalassemia, particularly of adult services.

In addition, we should not assume that patients have all of the facts either, and our survey revealed that there were significant gaps in their knowledge. Probing people with chronic conditions on their disease understanding and encouraging their questions is key, and proactively directing them to useful resources will support effective management and minimize the risk of future health problems.

Thalassemia management is rapidly evolving, with the advent of many novel therapies, and now is the time to change the way we engage with our patients. Further, the reliability of information available on the internet is inconsistent, and physicians must ensure that patients are provided with the most verified and up-to-date information. We hope this research serves as a call to action for physicians and patients that more can and must be done to improve patient care. The transfer of information plays a vital role, so that patients are empowered to achieve more and thrive.

Patient View

Thalassemia is a chronic disease, and its burden on patients and families is relentless. Pain and fatigue are a regular occurrence that affect daily life so much, with patients also juggling tests, scans and appointments with various health specialists. That's why health literacy in thalassemia is so important and the key to longevity, as a lack of understanding of the disease and its impact can have troubling consequences.

Ralph Colasanti, National President of Cooley's Anemia Foundation and thalassemia patient

Laurice Levine, Thalassemia patient and advocate

Living with thalassemia has taught us that understanding your condition and knowing where to find accurate information are essential to learning how to advocate for yourself. Improving health literacy is not easy – it's like struggling to find the correct path through a confusing maze of sometimes conflicting health information. It's time for the whole community to learn more, thereby improving clinical decisions on how the condition is managed. As patients, many of us have looked for physicians who are knowledgeable and who are willing to collaborate not only with us, but with specialists and other doctors on the team. So instead of 'being passengers on our health journey, we drive the car with our physicians'. Although global health system challenges impact already stretched physicians, they must prioritize seeking out further education on thalassemia and listening to their patients, as patients are experts regarding their health.

Patient View Continued

This white paper has been developed in collaboration with patients, caregivers, patient advocates and physicians, highlighting honest realities for our community. We need everyone's input to help solve the health literacy challenge in thalassemia. By shining a light on needs of persons affected by thalassemia, we can lift their voices to drive changes that make a real difference for them.

Although the thalassemia community worldwide has many needs, the common thread identified in the survey reflects the community's need for greater knowledge that can positively change their outcomes. With the right tools and knowledge, change is possible, and now we need bold efforts to help people with thalassemia recognize the role they can play in their own care.

We know personally that trying to understand health information can be daunting and confusing, especially when physicians don't have all the answers. But the survey shows us that most people with thalassemia have a desire to learn more. In the past, we relied on conferences or talking to other patients to get information, and as research has improved so has experts' understanding. Today, however, the spread of false information online is a common, worrisome problem. Social media forums are filled with people asking for advice because they don't understand something, can't access the correct information or don't know where to look. Luckily, there are published standards of care that are globally accepted and a patient community that's helpful and supportive at the national and international levels. Patient advocacy organizations (PAOs) do tremendous work, providing accurate information, and connecting patients to each other, to specialists, and to resources.

Once you better understand the condition, you can begin to understand its potential long-term impacts. Access to accurate and reliable medical information at your fingertips can be life-changing for thalassemia management, helping us to discuss and optimize our care.

It can be a full-time job navigating thalassemia, and it's valuable to learn more and talk to other patients about their experiences. Investing time in improving health literacy can change how people today and tomorrow can better manage or navigate their thalassemia for the better.

Executive Summary

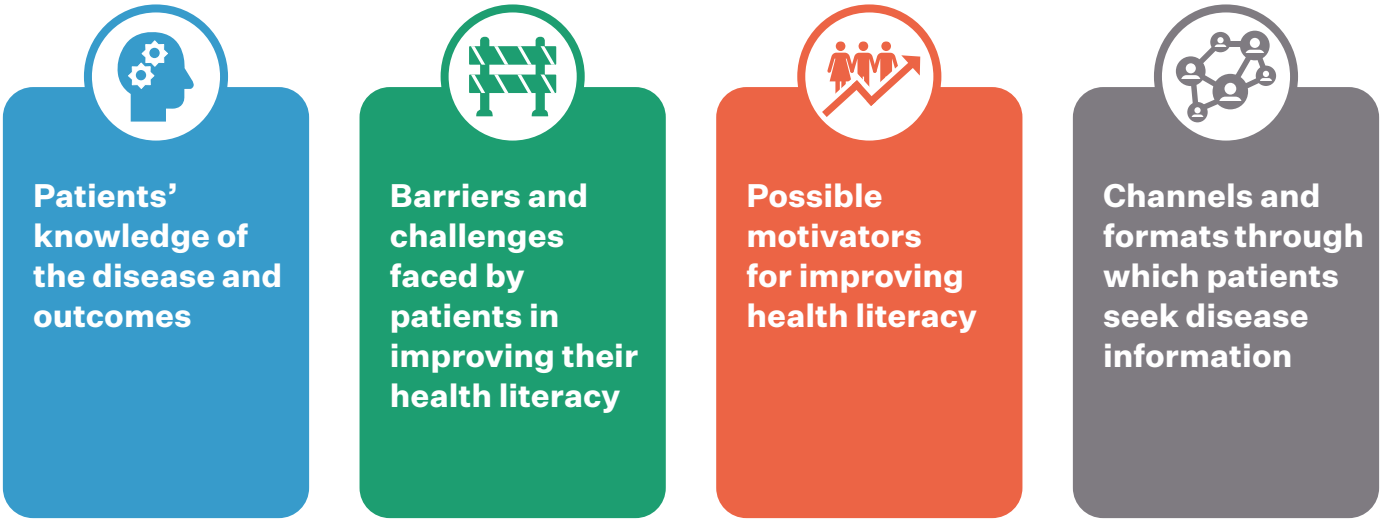
Thalassemia is an inherited blood disease affecting hemoglobin production that leads to anemia and a range of symptoms and complications that can severely impact daily life and long-term health if not properly managed.

Thalassemia is an inherited blood disease that affects the production of hemoglobin, the protein in red blood cells responsible for carrying oxygen throughout the body. The disease is categorized into two main types: alpha (α)-thalassemia and beta (β)-thalassemia, depending on which globin chain of the hemoglobin is affected. By disrupting hemoglobin production, thalassemia reduces the number of circulating red blood cells and shortens their lifespan, which leads to anemia and a range of symptoms and complications that can severely impact daily life and long-term health if not properly managed.[1–3]

Some individuals with thalassemia require regular transfusions (classified as transfusion-dependent thalassemia [TDT]), while others only need them intermittently or do not require them at all (classified as non-transfusion-dependent thalassemia [NTDT]).[4]

Thalassemia was initially mainly found around the Mediterranean and in Asia, but is now prevalent worldwide as a result of migration.[5] Knowledge and experience across different regions may vary based on the history of thalassemia in each region, cultural beliefs, and access to information, healthcare resources, and support.[5] Research on the long-term risks of thalassemia and recommended management, particularly in α-thalassemia and NTDT β-thalassemia, has evolved greatly in recent years.[5]

The Thalassemia AAC, an international group, which brings together a diverse range of voices from the thalassemia community, supported by Agios, was



Executive Summary Continued

established to better understand the unmet needs of the thalassemia community, and to support initiatives to improve patient outcomes and care.[6–8] In a needs analysis undertaken by the group, health literacy was identified as a key priority that could potentially contribute to people not following their treatment plans and different standards of care.[6–8] With the goal of broadening understanding of patient health literacy, the Thalassemia AAC defined four subtopics to investigate further (outlined on page 5).[8]

Based on these subtopics, the Thalassemia AAC developed a patient survey to better understand the international community's perspectives and to identify ways to potentially address health literacy needs and to support informed patient self-advocacy. In evaluating survey responses, it is important to take into consideration the possibility of participation bias, as people who agreed to participate in the survey are likely more engaged than the general patient population.

Spanning several geographic regions and thalassemia subtypes, findings from the survey have been presented at the 67th Annual Meeting and Exposition of the American Society of Hematology (ASH) in 2025,[9] with three main insights revealed:

- **INSIGHT 1: Patients generally report a high level of confidence in their knowledge of thalassemia, but there are clear gaps in their understanding of the disease, particularly regarding the hemoglobin level associated with increased rates of complications and the need for monitoring across all thalassemia types.** Physicians should be aware of existing knowledge gaps, encourage an open conversation, and proactively provide resources that can help patients understand their disease.
- **INSIGHT 2: Patients are confident in their physician's understanding and management of thalassemia and consider their physician a trusted source of information.** There is an opportunity to support providers with up-to-date information on the long-term complications of thalassemia and the need for monitoring so that they can provide the appropriate information to their patients.
- **INSIGHT 3: Patients would like to learn more about how to better manage their thalassemia, and the sources that they use to pick up new information are varied, showing the importance of reaching patients across different channels.** One important approach will be to support patients to have productive and proactive conversations with their physicians.

The majority of survey participants were confident in their understanding of thalassemia symptoms, treatment, complications, and lifestyle (Figure 3, page 17), and had trust in their physicians' management of their disease, with most (82%) considering their disease to be 'well managed' (Figure 8, page 21). Critically, however, many misidentified the threshold hemoglobin level associated with risk for complications, with more than half (53%) selecting 7 g/dL, and only 6% selecting the correct response of 10 g/dL (Figure 5, page 19). The findings suggest a need to examine how and where patients gain information about their disease. Disease education

resources must be accessible to more patients, in whatever format they wish to receive them, and physicians must support disease education efforts.

HCPs were identified by a majority of survey participants as their most trusted source of thalassemia information and an important motivator for seeking better care (Figure 13, page 29). However, up to 50% of participants reported obtaining information from sources other than their physicians, including internet search engines, social media, and other thalassemia patients (Figure 12, page 27). Participants indicated a desire to connect with and gain information from other patients with thalassemia, reminding us of the importance of a well-informed thalassemia community.

The phrase 'well managed' can mean different things to different people. For example, even with a majority of participants agreeing that their doctor manages their thalassemia well, and 91% rating their own ability to manage their condition as 'good', 'very good', or 'excellent' (Figure 6, page 20), most participants (66%) had a desire to learn more about how to manage their disease and thus be more active participants in their treatment decisions, including many who sought personalized treatment plans and greater support from their doctors (Figure 10, page 25). When asked about how their needs might change as they age, around half expressed an interest in gaining more information about the physical impacts of thalassemia and receiving referrals to other specialists (Figure 11, page 26).

Collectively, this study has led to important insights on the obstacles and challenges of improving health literacy in thalassemia, motivating factors for improving disease and outcomes knowledge, and where patients with thalassemia seek information. Findings from this study will inform efforts to improve disease education, promote better patient-to-patient and patient-to-physician conversations, and support health literacy efforts across the thalassemia community.

About the Thalassemia Advocacy Advisory Council (AAC)
Established in 2023, the AAC is an international, multi-disciplinary group of experts in thalassemia – patients, caregivers, patient advocates and physicians – fully supported and resourced by Agios. The group's vision is that people affected by thalassemia can collaborate to address educational needs and have their voices heard, no matter their disease subtype, background, or where they live.

Thalassemia AAC Members

Vision Statement

People affected by thalassemia can collaborate to address educational needs and have their voice heard, irrespective of disease sub-type, background, or geography.

1 **Sujit Sheth,** Pediatric Hematologist

2 **Ralph Colasanti,** Cooley's Anemia Foundation

3 **Craig Butler,** Cooley's Anemia Foundation

4 **Maria Domenica Cappellini,** Professor of Internal Medicine, Chief of the Rare Diseases Center

5 **Eleni Michalaki,** Thalassaemia International Federation

6 **Jill Deitrick,** Caregiver

7 **Lily Cannon,** Thalassaemia International Federation

8 **Laurice Levine,** Patient

9 **Dina Steagall,** ABRASTA – Brazilian Association of Thalassemia

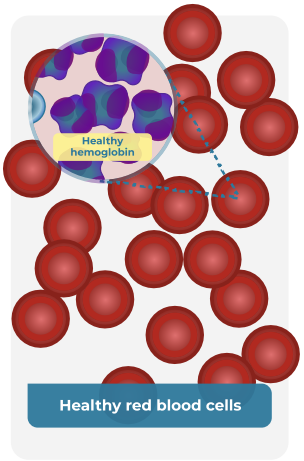
10 **Tobias Larkin,** Patient

Introduction

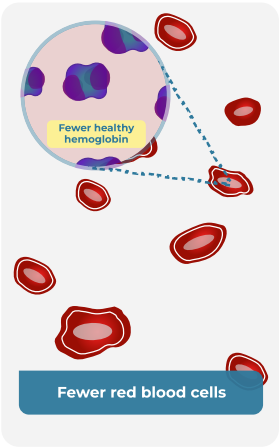
Thalassemia: An Under-Recognized Inherited Blood Disease

Thalassemia is an under-recognized inherited blood disease that reduces the number of circulating red blood cells and shortens their lifespan. It can look different from person to person, resulting in a variety of symptoms and complications that can substantially impact patient health-related quality of life and lead to severe comorbidities if not properly managed.[1–3]

To truly make sense of the community's challenges, we first must understand thalassemia and how it affects people and families around the world.



Blood not affected by thalassemia



Blood affected by thalassemia

Thalassemia is an inherited blood disease caused by reduced or absent production of one or more subunits of hemoglobin, which results in fewer healthy red blood cells being produced, with the cells that are produced circulating for shorter than normal lifespans.[10] More specifically, the imbalance in these subunits causes damage to the red blood cells and their precursors, leading to ineffective production ("erythropoiesis"), and premature destruction ("hemolysis") of red blood cells, which results in chronic anemia and related complications that can affect long-term health.[2,11]

Approximately 5% of the world's population are carriers of α-thalassemia, and 1.5% are carriers of β-thalassemia, meaning they carry a gene for thalassemia that they can pass on to their children, but do not have the disorder themselves.[10,12] In the past, patients with thalassemia have been found primarily in the Mediterranean basin, the Middle East, Southeast Asia and China; however, due to the migration of people over time, thalassemia is now seen worldwide, including in Northern Europe and the Americas.[5,13]

Introduction Continued

The severity of anemia in thalassemia can vary based on the number and type of genetic changes affecting the α-or β-globin genes.[14] Both α- and β-thalassemia share common symptoms primarily related to anemia, including fatigue (extreme and persistent tiredness), weakness and shortness of breath, and jaundice (yellowish discoloration of the skin). Complications can also occur across all types of thalassemia and can include brittle bones, iron overload, problems with the heart and circulatory system, liver disease and abnormal production of hormones. All patients are at the potential risk of serious complications if not properly monitored and managed, which can severely impact their long-term health and quality of life.[1,15,16]

How thalassemia affects someone depends on the person and usually can worsen with age, if not properly managed. This is thought to be due to both complications from the disease itself and also due to effects of some of the treatment approaches. Management of thalassemia has relied largely on blood transfusions, iron chelation, and splenectomy (removal of the spleen). These approaches collectively help to maintain hemoglobin levels and limit the severity of disease, but require specialized medical resources, long-term monitoring and can be accompanied by iron overload.[10] Thalassemia has more recently been classified as transfusion-dependent thalassemia (TDT) or non-transfusion-dependent thalassemia (NTDT). Patients with TDT are characterized by having early

presentation to clinical care, with severe anemia requiring regular and lifelong transfusion, whereas patients with NTDT do not depend on transfusions for survival but may receive them periodically to manage complications.[17]

The Thalassemia International Federation (TIF) has published guidance on the management of TDT β-thalassemia, NTDT β-thalassemia and α-thalassemia to aid physicians in clinical practice.[4,12,18] Clinical guidelines for the treatment of thalassemia vary by country depending on the local authorities and available resources in the area but are mostly based on adaptations of TIF's guidelines. However, many HCPs who treat thalassemia patients are not familiar with management standards, and coordinated care between primary care providers and specialists is often lacking.[19]

For patients, disease management can be a burden to daily routines or impose lifestyle changes that require behavior modifications to minimize risk for complications.[20] For patients with TDT, the burden of frequent blood transfusions and potential for adverse effects and complications can lead to poor treatment adherence, which can, in turn, add to worsening complications.[20] For patients with NTDT, blood transfusions may still be required on occasion or under specific circumstances (e.g., significant infection, pregnancy, surgery), with symptoms and complications being the best determinant of need for transfusion.[21] As such, monitoring for complications is important for preventive care and appropriate risk mitigation.[22] Studies have shown that patients with thalassemia are less likely to adhere to treatment when they don't understand that benefits of treatment outweigh the risks of nonadherence.[23] Conversely, level of disease knowledge positively correlates to treatment adherence.[23]

Introduction Continued

With the objective to better understand and define unmet needs of patients with thalassemia and their HCPs, the Thalassemia AAC identified health literacy (the understanding of the disease, its complications, and treatment approaches) as a key unmet need that can contribute to poor adherence to treatment recommendations and varying standards of care for patients with thalassemia.[6,8] With the goal of broadening understanding of patient health literacy, the Thalassemia AAC developed an international patient survey to gain insights into the community's perspectives and to identify new ways to address health literacy needs and support informed patient advocacy.

Survey Process

A 15-minute online survey was developed to understand the impact of health literacy on disease management among patients with thalassemia.

The aim of the survey was to describe the level of health literacy in patients with thalassemia, or more simply, patients' understanding of disease management and its long-term impacts.

This translated into the research question that guided the survey: Among patients with thalassemia, what is their level of health literacy, i.e., how much of their disease management and long-term impacts of the disease do they understand? And what resources do they use, and where do they seek and find their disease information?

Survey Focus
Topics covered by survey questions are listed in the figure below.

1. Screening	To gather information and assess survey eligibility
2. Demographics	Optional questions to better understand the participants
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 improved understanding and better self-management of the disease

Survey Process Continued

The survey was conducted among thalassemia patients who met the following criteria:

- Physician-diagnosed thalassemia of any subtype, excluding patients who have been diagnosed with α- and β-thalassemia trait.
- Aged 18 or older.
- Agreed to participate in the study.
- Able to provide informed consent.
- Were not taking part in an Agios-sponsored clinical trial.

Two methods were used to recruit participants:

- Phase 1: Agency-based recruitment and data collection were conducted by Vitreous World between March 15 and April 17, 2024. Countries included the USA, Brazil, Italy, Greece, and the United Arab Emirates (UAE).
- Phase 2: Patient organization recruitment was completed by TIF between August 1 and October 31, 2024. Countries included Brazil, Kuwait and Saudi Arabia.

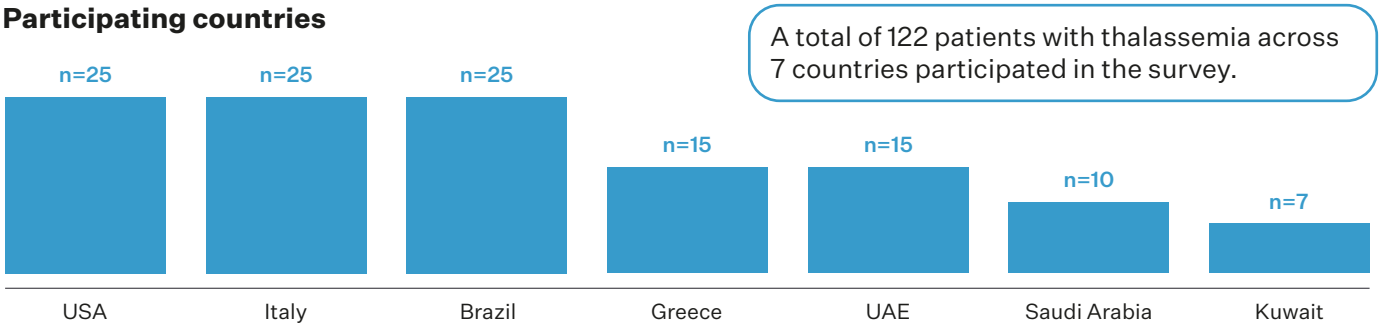
The survey was conducted according to British Healthcare Business Intelligence Association (BHBIA) Legal and Ethical Guidelines, as well as guidelines established by the UK's Market Research Society (MRS). Survey information was handled in accordance with Agios Data Privacy Notice, country-specific regulations, and the General Data Protection Regulation (GDPR) guidelines where appropriate.

Who Answered the Survey

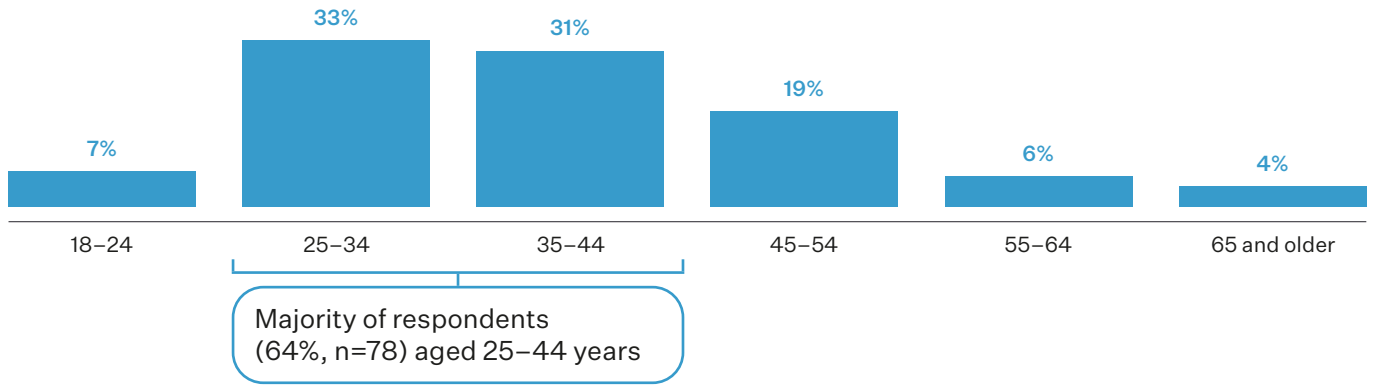
A total of 122 patients with thalassemia from the USA (n=25), Italy (n=25), Brazil (n=25), Greece (n=15), the UAE (n=15), Saudi Arabia (n=10), and Kuwait (n=7) participated in the survey. The majority were 25–44 years of age, more than half were female, and most had completed a bachelor's degree or higher (Figure 1). A diagnosis of β-thalassemia was more common than α-thalassemia. More than half of participants received blood transfusions every 2 or 3 weeks, and the most common frequency of physician visits was every 2 weeks or monthly (Figure 2).

FIGURE 1: DEMOGRAPHICS OF SURVEY PARTICIPANTS

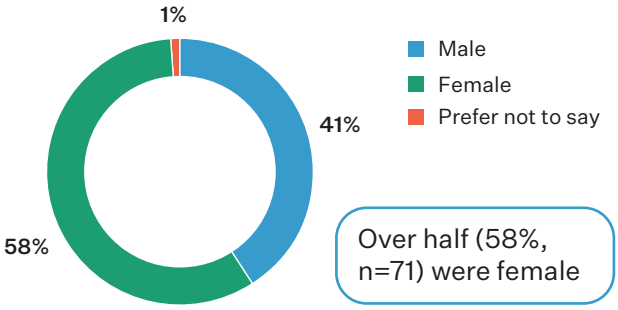
Participating countries



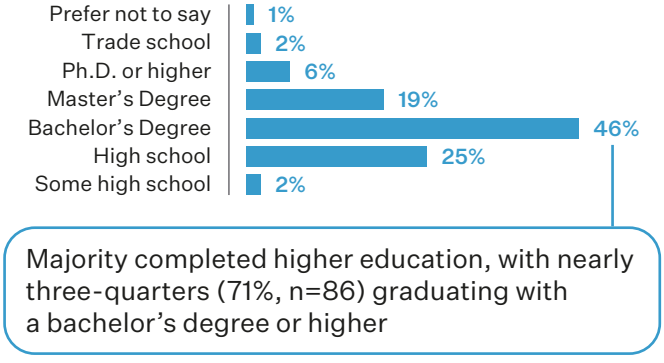
Age (years)



Gender



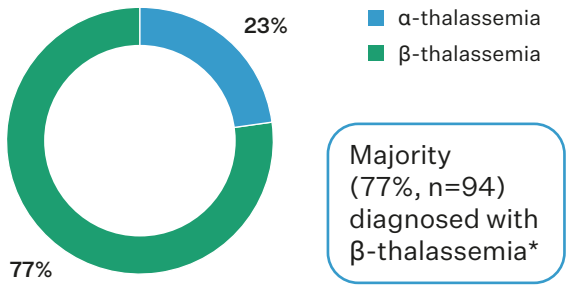
Highest degree of education completed



Who Answered the Survey Continued

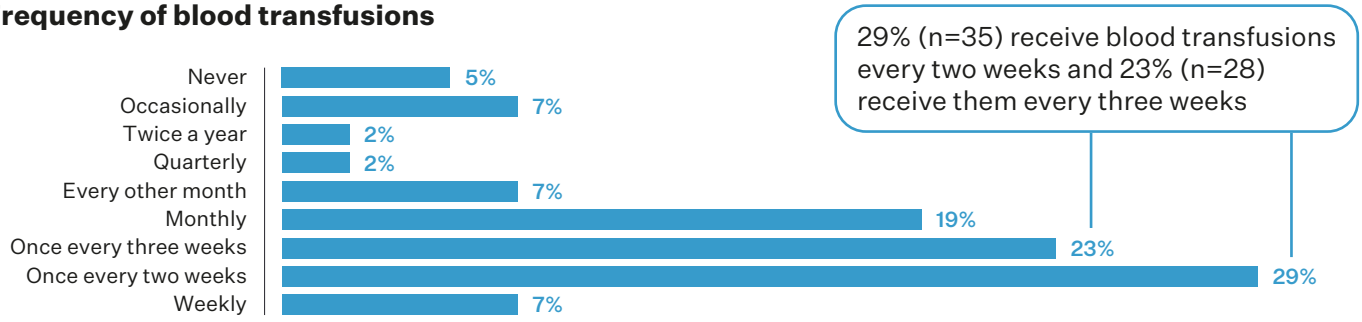
FIGURE 2: DISEASE CHARACTERISTICS AND DOCTOR VISITS

Thalassemia diagnosis

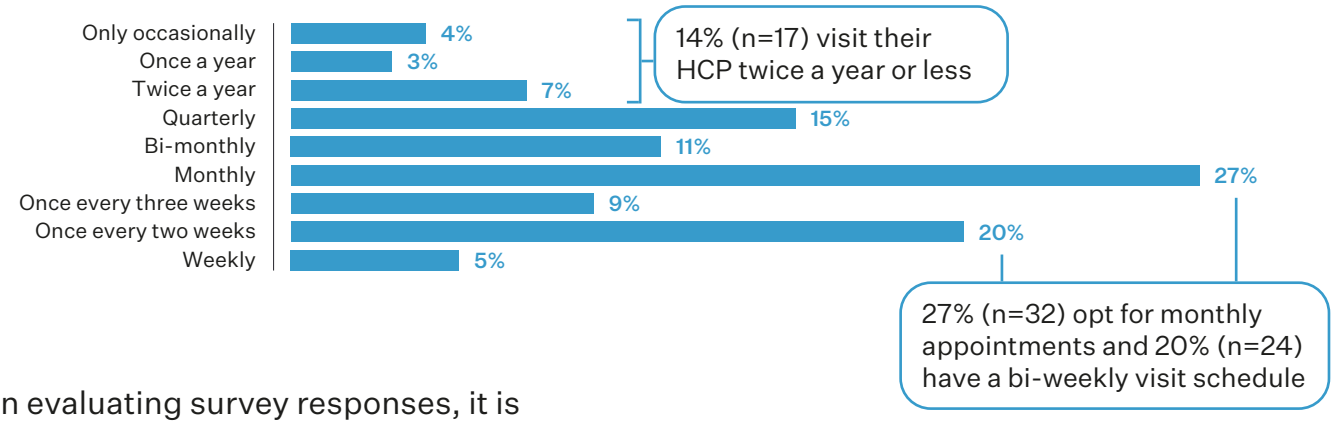


* Including both transfusion-dependent and non-transfusion-dependent thalassemia.

Frequency of blood transfusions



Frequency of seeing thalassemia-treating health care provider



In evaluating survey responses, it is important to take into consideration the possibility of participation bias, as people who agreed to participate in the survey are likely more engaged with their thalassemia care than the general patient population.

Findings

INSIGHT 1: Patients generally report a high level of confidence in their knowledge of thalassemia, but there are clear gaps in their understanding of the disease, particularly regarding the risks of complications and need for monitoring across all thalassemia types.

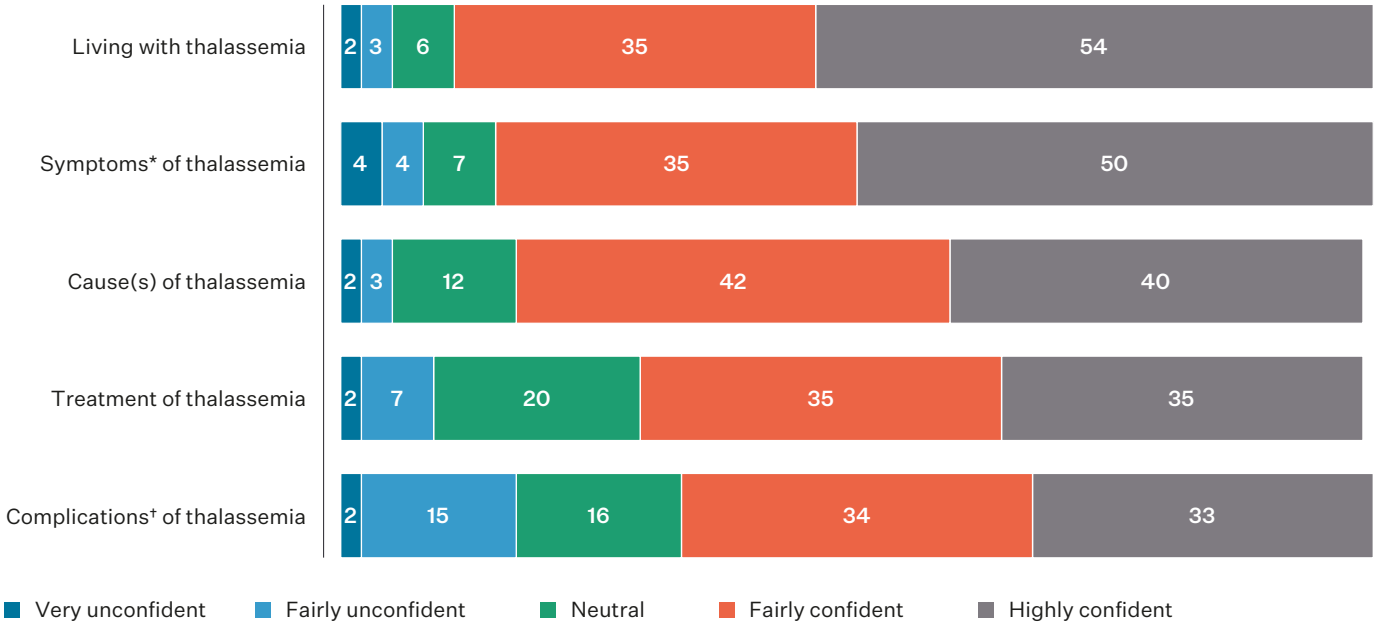
Overall, survey participants reported feeling confident in their knowledge of thalassemia, with the majority stating that they are highly or fairly confident regarding their understanding of the symptoms of thalassemia (85%) and living with the disease (89%).

Areas to build confidence include thalassemia complications and treatment of thalassemia, as around one-third of participants rated themselves not confident/neutral in their knowledge of these topics (Figure 3).

FIGURE 3: CONFIDENCE IN THALASSEMIA KNOWLEDGE

Question: How confident, or unconfident, are you in your knowledge about these aspects of thalassemia?

Level of confidence on (percentage of respondents)



Percentages may not total 100% due to rounding.

* A symptom is a physical or mental manifestation of thalassemia apparent to the patient i.e., fatigue or pale skin. †Complications generally involve a worsening in the severity of the disease or the development of new signs, symptoms, or pathological changes i.e., infections, heart disease.

Findings Continued

That said, nearly all participants surveyed understand that not treating thalassemia could lead to serious complications in the future (Figure 4). And, encouragingly, almost half of participants (43%) stated that their knowledge of thalassemia had improved a lot over the previous five years.

Worryingly, however, the survey data reveal a potential overestimation of disease understanding. Only 6% of participants correctly recognized that a hemoglobin level of ≤10 g/dL is associated with an increased risk of complications. Additionally, most participants (53%) incorrectly selected a threshold of ≤7 g/dL, suggesting patients do not fully understand their disease. Fourteen percent reported they did not know or were unsure (Figure 5).

"For me as a patient with alpha thalassemia, good management has meant hematologists pushed me to do annual monitoring—MRIs, EKGs, echocardiograms, DEXA scans, and more—to check for iron overload and other complications. Not every alpha thalassemia or NTDT patient has access to that kind of guidance, though, so many simply don't know what comprehensive care should look like."

Tobias Larkin,
Patient

FIGURE 4: UNDERSTANDING THE CONSEQUENCES OF IMPROPER THALASSEMIA TREATMENT

Question: As far as you know, do you agree, or disagree, with the following statement: if not treated properly, thalassemia can lead to serious complications that may require treatment in the future?

Level of agreement

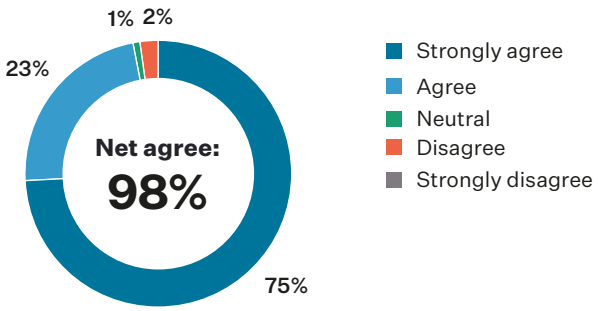
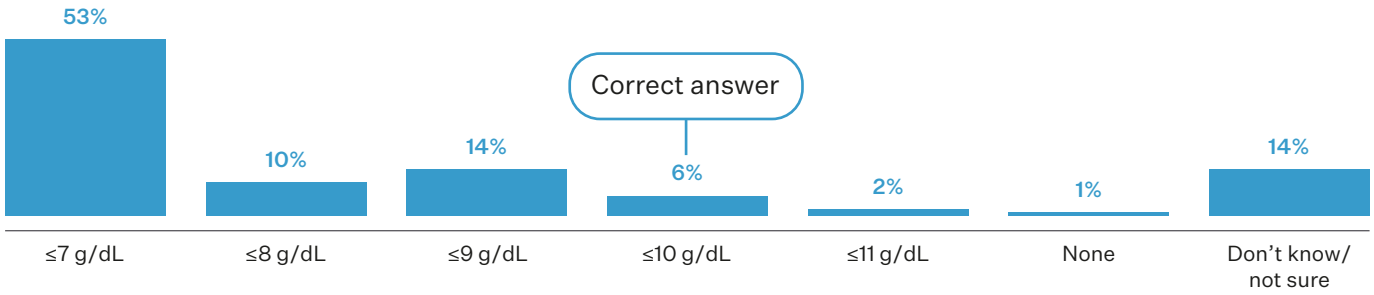


FIGURE 5: UNDERESTIMATION OF HEMOGLOBIN LEVELS ASSOCIATED WITH COMPLICATIONS OF THALASSEMIA

Question: What hemoglobin level is most associated with an increased risk of developing complications from thalassemia? Select one.

Hemoglobin levels associated with complications



"The fact that so many patients feel confident about managing their thalassemia, while at the same time being unaware of monitoring requirements for alpha thalassemia and NTDT, highlights a major knowledge gap. As an alpha thalassemia patient, I'm not surprised by this. In my own experience, new doctors have sometimes assumed I had beta thalassemia, which shows how much the focus on transfusion-dependent beta thalassemia can overshadow other forms of the disease."

Tobias Larkin,
Patient

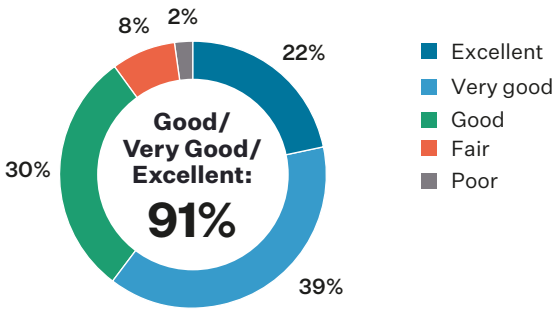
Despite this, 91% of participants rated their ability to manage their condition as good, very good or excellent (Figure 6), indicating a positive outlook and high level of confidence. However, this result contrasts with another finding in the survey which indicated that respondents regularly struggle with fatigue (i.e., tiredness and weakness) (Figure 7), potentially revealing a disconnect between patients' expectations for their disease management versus what optimal disease management could look like.

Findings Continued

FIGURE 6: ABILITY TO MANAGE THALASSEMIA EFFECTIVELY

Question: Thinking specifically about disease management, how would you describe your ability to manage your thalassemia effectively?

Effectiveness of thalassemia management



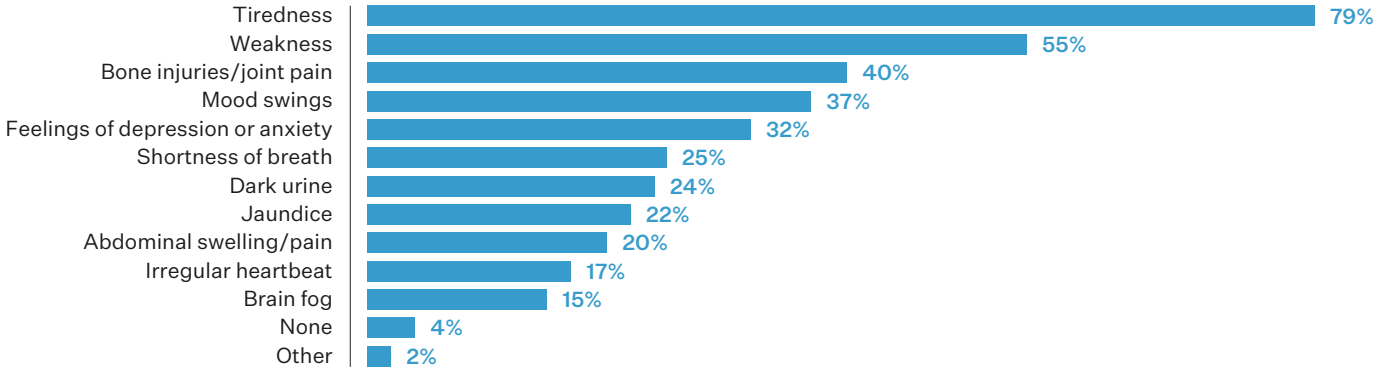
"It's truly encouraging that so many people feel confident in their ability to manage thalassemia. However, the fact that fatigue and tiredness remain common challenges shows there's still room to improve care and support. This highlights an important opportunity—for our community to work together to help people with thalassemia not only feel empowered, but also achieve an even better quality of life through more optimal disease management."

Craig Butler,
Cooley's Anemia Foundation

FIGURE 7: PHYSICAL AND EMOTIONAL IMPACTS EXPERIENCED ON A REGULAR BASIS

Question: What are the physical and emotional impacts you experience on a regular basis (i.e., daily or weekly)? Select all that apply.

Physical and emotional impacts experienced regularly



INSIGHT 2: Patients are confident in their physician's understanding and management of thalassemia and consider their physician a trusted source of information.

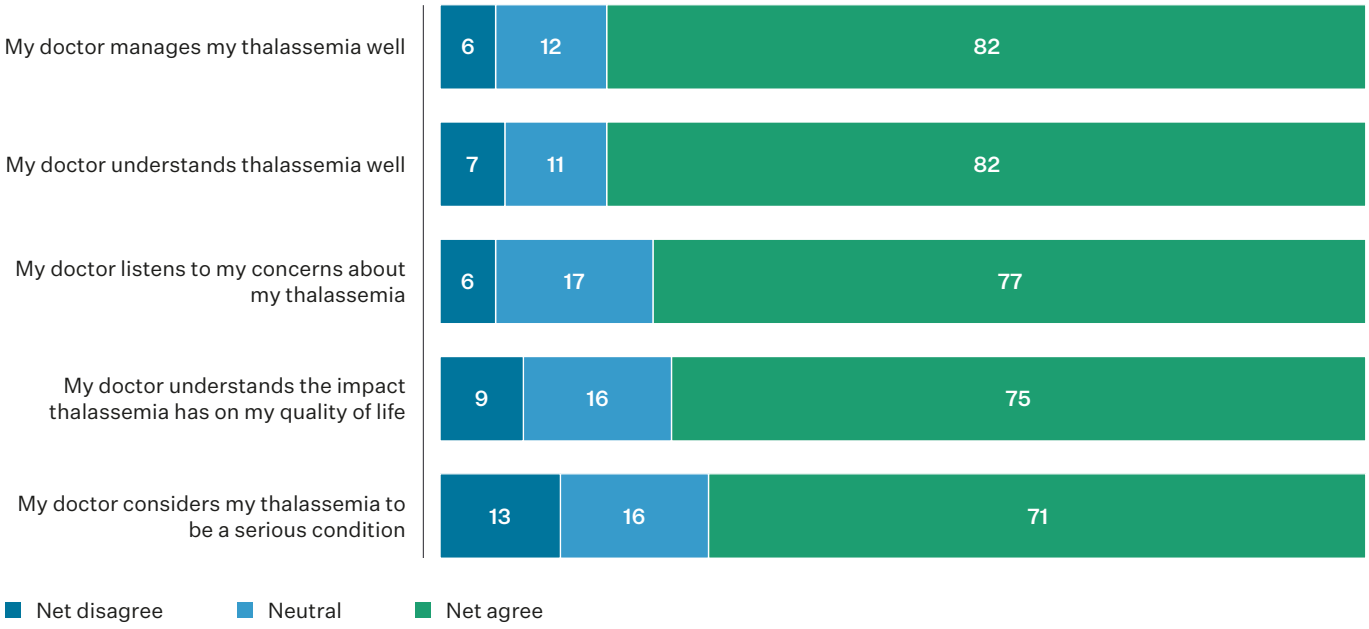
This points to an opportunity to educate physicians on the long-term complications of thalassemia, the need for monitoring and to support them with patient education resources on this topic.

More than four-fifths of participants agreed that their thalassemia-treating doctor understands their condition well (82%) and manages it effectively (82%). Nearly four in five (77%) agreed that their disease-related concerns are heard by their thalassemia-treating doctor, while nearly three in four reported that their condition's significance is understood (71%) (Figure 8).

FIGURE 8: RESPONDENTS' ASSESSMENT OF THEIR DOCTORS' UNDERSTANDING AND MANAGEMENT OF THALASSEMIA AND ITS IMPACT

Question: Thinking about your thalassemia-treating doctor, how much do you agree, or disagree, with the following statements?

Level of agreement (percentage of respondents)



Findings Continued

In addition, two-thirds of patients (67%) identified HCPs as their most trusted source of information (Figure 9).

A separate/follow-up question revealed that a resource being 'clear' and 'relevant to the respondent's experience' are key in driving patients' trust in an educational resource. Participants' use of HCPs as a trusted source of thalassemia information suggests that efforts to support physicians with patient education may provide an opportunity to help address these knowledge gaps.

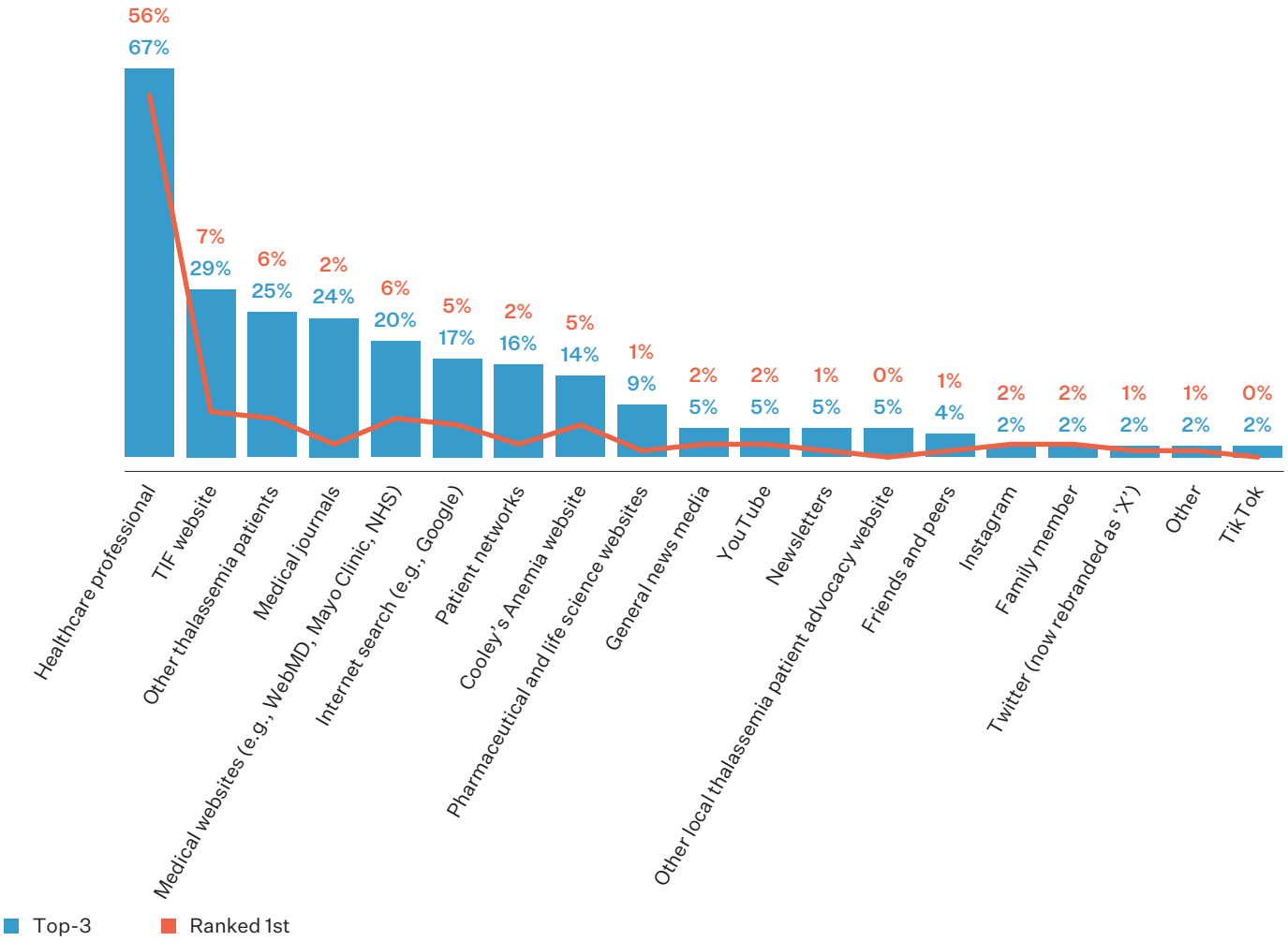
"The global thalassemia community is incredibly diverse, as are the unmet needs of patients. Yet one constant remains: high-quality care depends on expert physicians equipped with up-to-date scientific and medical knowledge. This expertise builds trust and confidence from patients and families, supporting adherence and better long-term outcomes. TIF's Guidelines, developed by international medical experts since 1999 and adopted by the World Health Organization (WHO) have bridged knowledge gaps and set standards for consistent care, with many countries adapting them to local realities. Offered in multiple formats to ensure practicality for physicians, they strengthen physician-patient alignment, empowering patients to take a more active role in the management of their disease, participating in meaningful and productive discussions, as equal partners in their treatment journey."

Lily Cannon, Thalassaemia International Federation

FIGURE 9: THALASSEMIA INFORMATION SOURCES, MOST TO LEAST TRUSTED

Question: Among the channels or sources that you use regularly to search for information about thalassemia and/or its management, which do you trust the most? Please rank from most to least trusted.

Trusted sources



Blue bars represent the percentage of respondents that ranked a source as one of their three most trusted sources for thalassemia information, while the line represents the percentage of respondents that ranked a source first. For example, in the chart above, 29% of respondents ranked the TIF website as one of their top most trusted sources, while 7% ranked it first.

Findings Continued

"Thalassemia patients around the world place a high level of trust in their doctors, with whom they develop a therapeutic alliance. This is a close and long-term relationship that requires knowledge, involvement, time, and personalized care to manage the underlying disease and its complications. Educating doctors and patients is crucial for better management of thalassemia."

Eleni Michalaki, Thalassaemia International Federation

INSIGHT 3: Patients would like to learn more about how to better manage their thalassemia, and the sources that they use to pick up new information are varied, showing the importance of reaching patients across different channels.

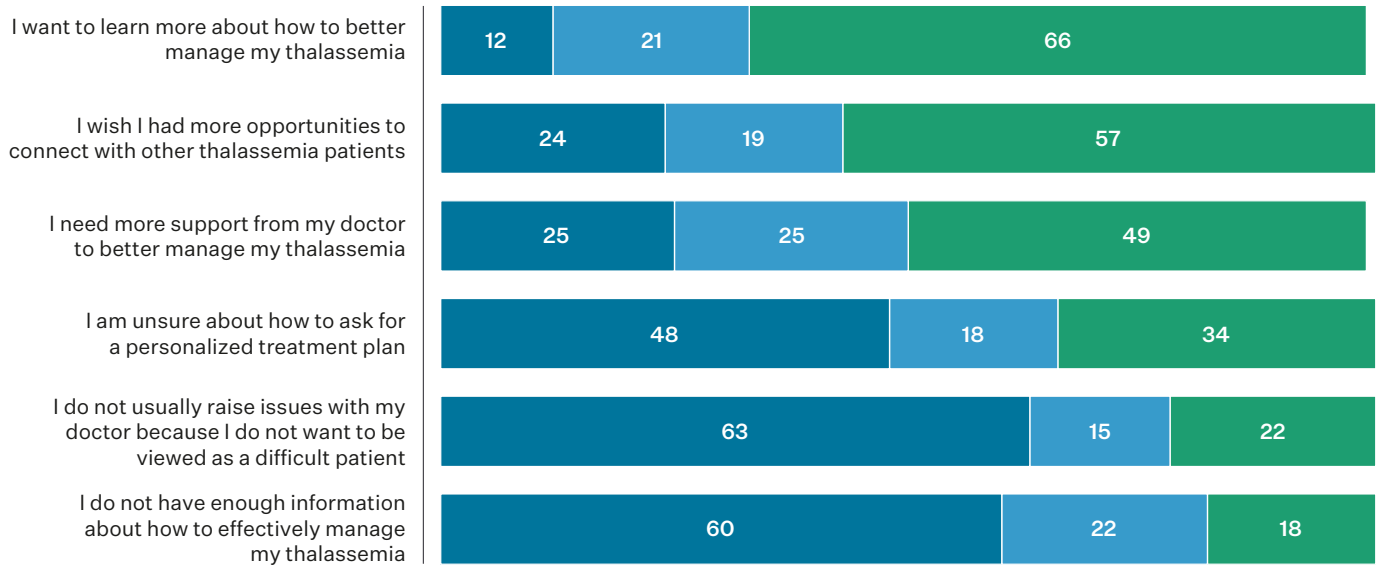
One important approach will be to support patients to have productive and proactive conversations with their physicians.

Survey participants wanted to learn more about how to better manage their thalassemia (66%), but some were unsure about how to ask for a more personalized treatment plan (34%) (Figure 10). More than half (57%) wished for more opportunities to connect with other thalassemia patients—achievable through both patient advocacy organizations and informal community connections—and about half (49%) said they need more support from their doctor to better manage their thalassemia (Figure 10). Given that the physician was the top trusted source of information for most participants (67%; Figure 9), there is an opportunity to support patient-physician conversations about optimizing treatment plans based on the latest evidence.

FIGURE 10: NEED FOR COMMUNITY SUPPORT AND PRACTICAL GUIDANCE ON MANAGING THALASSEMIA

Question: How much do you agree or disagree with the following statements?

Level of agreement (percentage of respondents)



Percentages may not total 100% due to rounding.

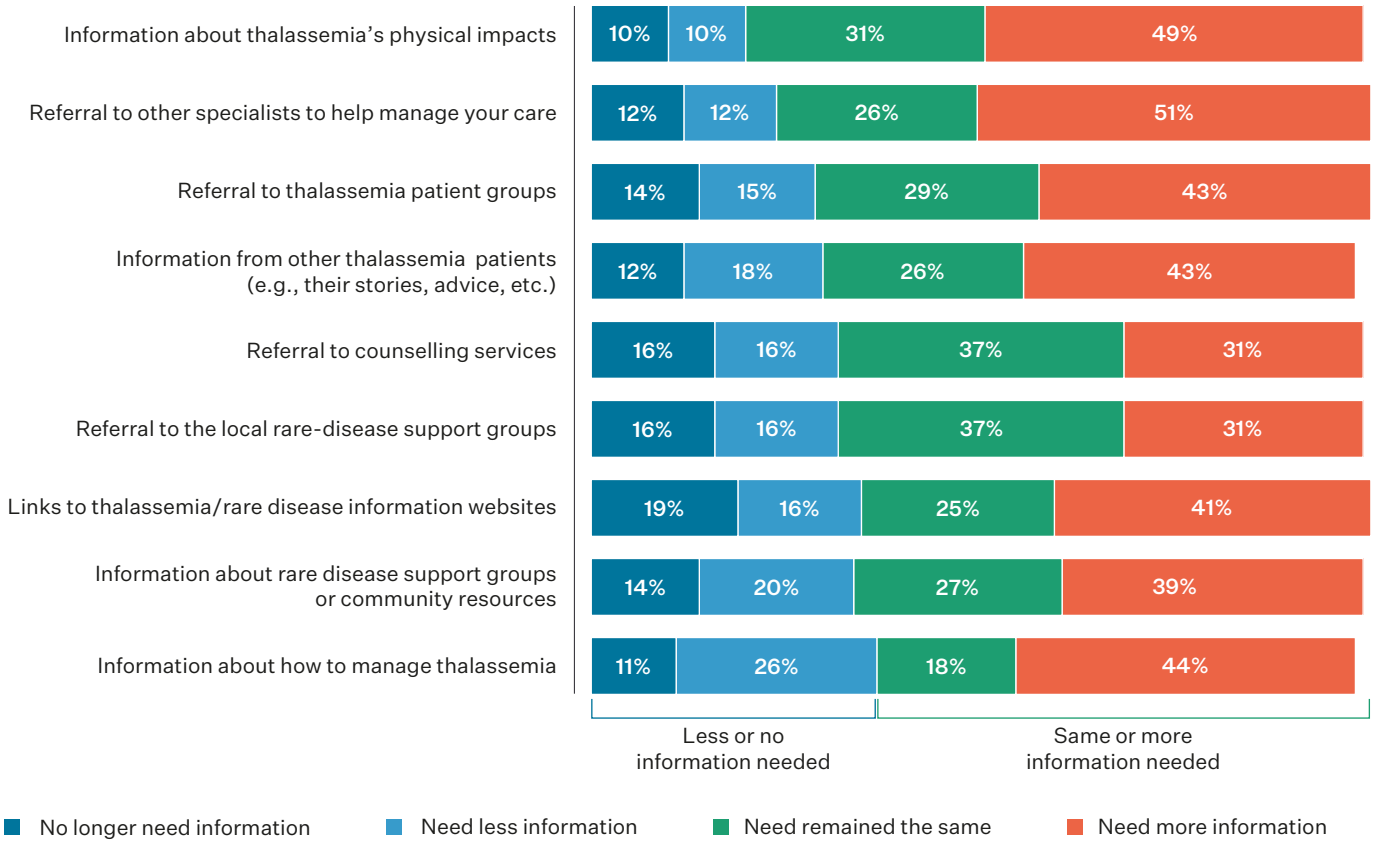
Findings Continued

Additionally, the survey indicated that approximately half of participants need more information on how to manage the physical impacts of their condition and referral to specialists as they get older, indicating that educational and care needs change over time (Figure 11).

FIGURE 11: INFORMATION AND SUPPORT NEEDS WITH AGING

Question: As you have gotten older, what information and support have you needed more of, and have you needed less of?

Changing needs with aging



Percentages may not total 100% due to rounding.

A wide variety of sources were used by survey participants to obtain their information, highlighting that patients have different preferences and that there are lots of sources of information available. Participants reported sources including internet search engines, social media, peers, and HCPs. Patient-focused information sources were favored, with half of participants preferring to seek information from other patients (50%), indicating a strong reliance on community support (Figure 12). Additionally, 57% of participants indicated they want more opportunities to connect with other patients, further highlighting a desire to connect with the community to learn more (Figure 10).

Live meetings/conferences (49%) and videos (48%) were the preferred ways for patients to receive disease-related information (Figure 12). Continuing to offer disease education in different formats, with a focus on physicians, other patients and the community as common sources, may support closing these gaps in disease understanding.

FIGURE 12: THALASSEMIA INFORMATION SOURCES: SOURCES REGULARLY USED AND PREFERRED CONTENT FORMAT

Question: Which, if any, of the following channels do you regularly use to find information about thalassemia and/or its management? Select all that apply.

Sources regularly used

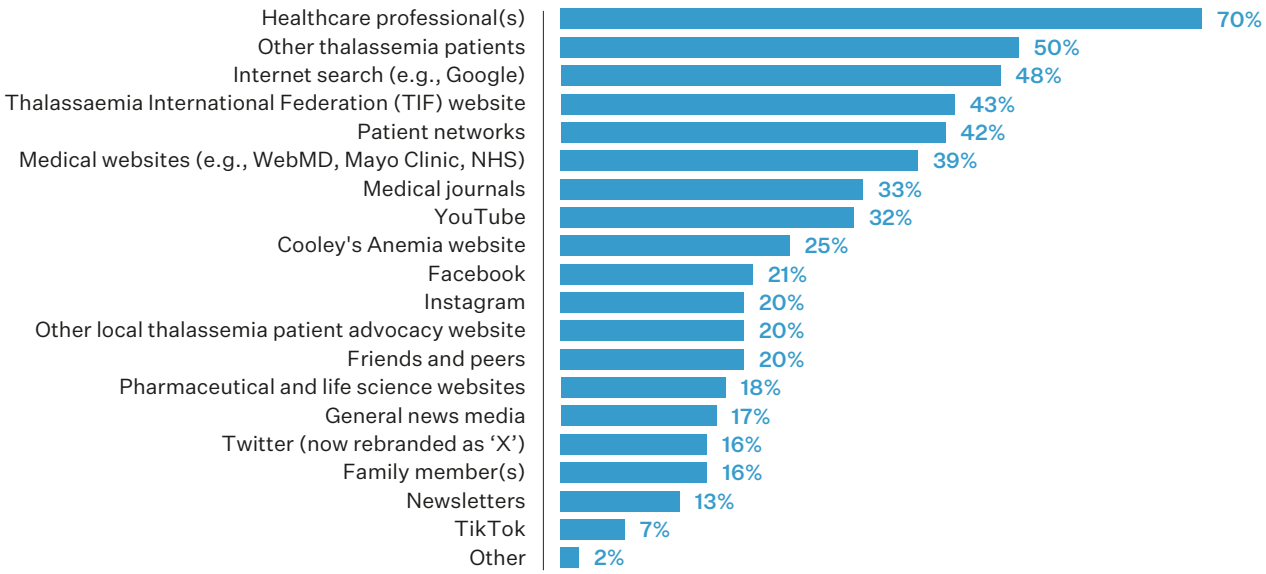


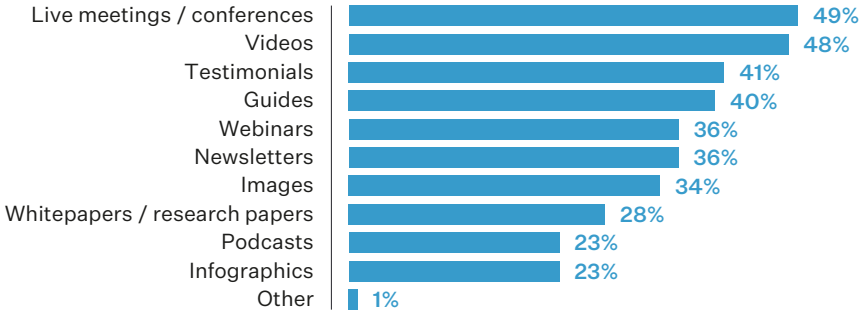
Figure 12 continued on next page

Findings Continued

FIGURE 12 (continued): THALASSEMIA INFORMATION SOURCES: SOURCES REGULARLY USED AND PREFERRED CONTENT FORMAT

Question: Now, thinking about thalassemia-related content, what format(s) do you prefer to receive that information in? Select all that apply.

Preferred content format

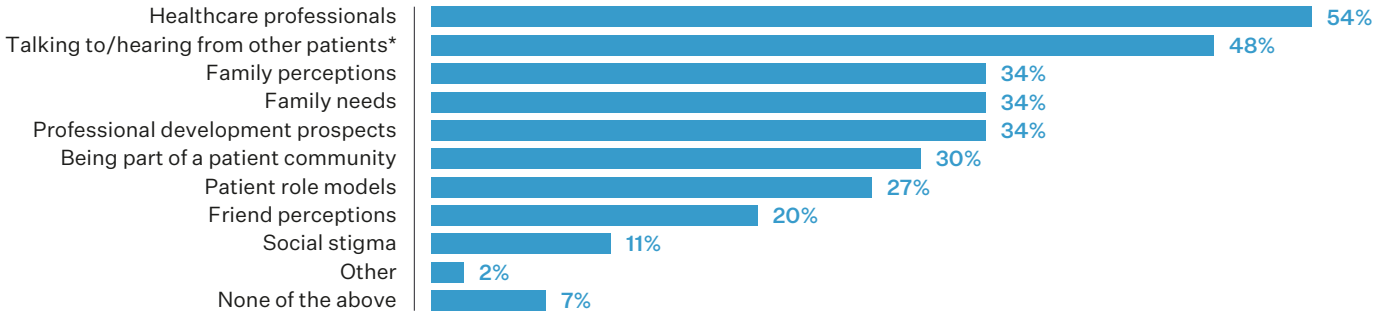


In terms of what motivates patients to learn more, speaking to HCPs and other patients (whether that is for advice or to hear about their experiences) appears to be key for patients when seeking better care for their condition. A third of participants said their family needs (34%) and perceptions (34%) play a role in motivating them to seek better care (Figure 13). In addition, being part of a thalassemia patient community was an encouraging factor for a majority of the participants (82%) (Figure 14).

FIGURE 13: MOTIVATING FACTORS FOR SEEKING BETTER THALASSEMIA CARE

Question: Has any of the following motivated you to seek better care for your thalassemia? Select all that apply.

Motivators for better care

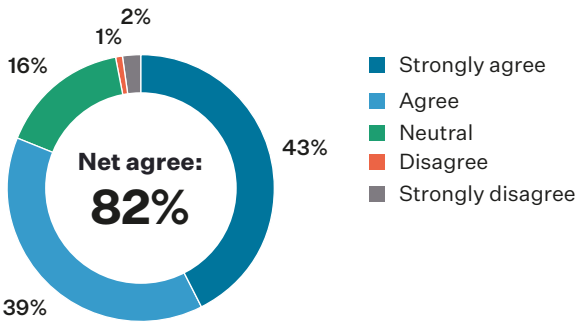


* e.g., stories, experiences, advice, etc.

FIGURE 14: ROLE OF THALASSEMIA PATIENT COMMUNITY IN MOTIVATION

Question: How much do you agree or disagree with the following statement: "being part of a thalassemia patient community motivates me/would motivate me to learn more about my disease"?

Level of agreement



"As a caregiver, I've seen firsthand how important it is for people with thalassemia and their families to connect with others who truly understand what they're going through. Speaking with HCPs is crucial but hearing from other families in the same position as ours has been truly invaluable for us. Being part of this community not only helps us learn more about the condition, but it also gives us confidence and strength to keep pushing for the best possible care."

Jill Deitrick,
Caregiver

Conclusions

Thalassemia is an under-recognized inherited blood disease that affects people around the world. It affects the production of hemoglobin, leading to anemia and a range of symptoms and complications that can severely impact daily life and long-term health.[1–3] However, poor knowledge of long-term complications can lead to poor adherence to recommended management approaches from HCPs and lack of monitoring for those complications (particularly in NTDT).[23] Meanwhile, regional differences in hospitals and clinics, local policies, access to healthcare resources, and the expertise of treating physicians can lead to differences in standards of care. There is a need for improved education and self-advocacy to support appropriate disease management.[5]

Health literacy was identified by the Thalassemia AAC as a critical unmet need in patients with thalassemia, and a survey was developed to understand patients' current knowledge of thalassemia and its outcomes, identify obstacles and challenges faced by patients in improving their own health literacy, determine potential motivators to improve health literacy, and to analyze where and how patients most effectively gain information about their disease. The survey of 122 thalassemia patients from North America, Europe, South America, and the Middle East, revealed three main insights, unlocking new possibilities to improve the patient experience:

INSIGHT 1: Patients generally report a high level of confidence in their knowledge of thalassemia, but there are clear gaps in their understanding of the disease, particularly regarding the hemoglobin level associated with increased rates of complications and the need for monitoring across all thalassemia types. Physicians should be aware of existing knowledge gaps, encourage an open conversation, and proactively provide resources that can help patients understand their disease.

INSIGHT 2: Patients are confident in their physician's understanding and management of thalassemia and consider their physician a trusted source of information. This points to an opportunity to educate physicians on the long-term complications of thalassemia, the need for monitoring and to support them with patient education on this topic.

INSIGHT 3: Patients would like to learn more about how to better manage their thalassemia, and the sources that they use to pick up new information are varied, showing the importance of reaching patients across different channels. One important approach will be to support patients to have productive and proactive conversations with their physicians.

So, what next?

We are in a time of rapid change, with advances in disease knowledge and the potential introduction of new therapeutic approaches that could change the face of thalassemia care. It is important for patients to be conscious of the long-term effects of their disease, thereby enabling them to make informed decisions about their treatment and care.

Participants' use of HCPs as a trusted source of thalassemia information suggests that physicians can play a key role in addressing patient knowledge gaps, and they need to be ready. Increased collaboration between those involved and enhanced awareness of PAOs among patients and physicians will improve patient knowledge of their disease now and for future generations and help patients to positively impact their own care.

"The results of this global survey highlight both progress and persistent gaps in thalassemia care. While many patients feel confident in their knowledge and trust their physicians, the findings reveal critical areas where awareness of complications and monitoring remains limited. For TIF, these insights reinforce the urgent need to strengthen health literacy, equip physicians with updated resources, and ensure patients everywhere have access to accurate, practical information. By closing these gaps, we can improve adherence, reduce complications, and empower people with thalassemia worldwide to play an active role in their care and achieve better long-term outcomes."

Lily Cannon, Thalassaemia International Federation

Calls to Action

- **For Patient Advocacy Organizations (PAOs)**

1. Combat misinformation passed between patients or from unreliable sources
2. Continue to proactively share accurate health information in different ways and formats to build people's knowledge
3. Encourage open and honest conversations between patients and HCPs about thalassemia and its management

- **For Healthcare Professionals (HCPs)**

1. Access, understand and follow the relevant TIF and/or local/regional guidelines for the appropriate management of patients with thalassemia; share this knowledge with the wider clinical team
2. Correct misinformation shared by patients; repeat and reinforce accurate information
3. Become familiar with, and proactively direct patients to the educational resources on PAO websites, in particular around threshold hemoglobin levels of concern and risks of long-term complications across all thalassemia types regardless of transfusion status
4. Understand the differences in patient needs between thalassemia subtypes (α- and β-thalassemia; TDT and NTDT) and other demographic subgroups (sex, age, race), and be prepared to educate on the potential complications of each

- **For patients and caregivers**

1. Be curious about thalassemia and build an understanding of the disease and its complications
2. Speak up for yourself or a loved one regarding concerns about thalassemia management and changing needs with aging
3. Share experiences or educational resources with others in the thalassemia community
4. Correct misinformation passed between patients or from unreliable sources; direct to easy-to-understand resources on the TIF and Cooley's Anemia Foundation websites

- **For the pharmaceutical industry**

1. Support the development of health education tools in alternative formats and languages that can be proactively used to support patients and improve health literacy
2. Partner with PAOs and physicians to spread awareness of updates in guidelines and research milestones in thalassemia

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