ThalassemiaNEWS

A quarterly newsletter keeping the medical and patient community in touch with thalassemia developments

Issue 1 2025

IN PROFILE

An in-depth view of an organization or individual involved in thalassemia

In this Issue, we bring you excerpts from an interview with Holly John, Head of Patient Advocacy at Agios Pharmaceuticals Inc.

Holly John
Head of Patient Advocacy

Can you briefly describe your role as Head of Patient Advocacy at Agios?

My role, as well as that of my team, is to bring the voice of the patient into everything we do at Agios. By ensuring our colleagues understand the lived experience of patients and other members of the community, we make better decisions and develop more impactful solutions. And by partnering with patients, we're helping to unify and empower their collective voice, driving real change.

How did you come to patient advocacy at Agios?

I've had a long career in the pharmaceutical industry and have held positions in both Commercial and Medical Affairs. But it was only when I started working with rare disease patients that I felt I'd found my true calling. Through them, I've gained an appreciation for the vitality of these communities and an understanding of the power of speaking with one voice. It's a real privilege to be able to be able to give back to patients even a small portion of what they've given me.

What are you most proud of since you've been at Agios?

I'd say that I'm most proud of Agios adopting the Patient Ally mindset. By working with patients, caregivers, and physicians to develop that concept, we established a company vision that we can rally behind. And I have to say, everyone at Agios has embraced this idea, and it has changed how we do things – we give patients a seat at the table, actively listen to their input, act on it, and then communicate about what we've done. A lot of companies talk about doing this, but few 'walk the talk'.

What do you find most challenging in the role?

Our main challenge is that there simply aren't enough hours in the day! We get a lot of positive feedback from the patient community that we are making a difference, but that's tempered with the knowledge that more needs to be done. For example, I've learned that the broader healthcare system – including the pharma industry – have an opportunity to better understand the health literacy gaps that may exist due to multicultural and language challenges. This can hinder information related to research and clinical information reaching those that need it most. I recognize that patients provide us with a window into the most personal aspect of their lives, and I feel an enormous responsibility to help close this health literacy gap.

What inspires you the most?

I am inspired when I see patients form meaningful connections – I've seen that simple act lead to transformative change in their lives. At Agios, our Patient Committee Ambassadors are working hard to develop and support networks via a grassroots approach, and it is starting to make a difference. For example, after attending one of our local events, a patient wrote us that, "I was unseen, unheard and forgotten, but today I have found my voice and my people". Impact like that motivates me every day!

KEY DATES

May 7th-10th 2025 Louisville, USA
American Society of Pediatric Hematology/Oncology (ASPHO)

May 5th-6th 2025 Rome, Italy
Pioneering Advances in Hematology: Bridging Research and Clinical Practice.

May 8th, 2025
International Thalassaemia Day

June 12th-15th, Milan, Italy
30th European Hematology Association Congress

July 7th-8th 2025 Zurich, Switzerland
23rd World Hematology Congress

July 11th-13th 2025 Washington, USA
1st TIF Pan American Conference on Thalassaemia & Other Haemoglobin Disorders and the 2nd RAIN Summit

September 29th-30th 2025 Vienna, Austria
4th European Congress on Hematology and Blood Disorders

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The newsletter content is prepared by thalassemia experts in collaboration with Agios Pharmaceuticals.
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Here we present an in-depth profile of one of the largest thalassemia advocacy organizations globally, courtesy of a behind-the-scenes interview with Craig Butler, National Executive Director at Cooley's Anemia Foundation.

Craig Butler
National Executive Director

Cooley's Anemia Foundation (CAF)

Cooley's Anemia Foundation

https://thalassemia.org/

The Cooley's Anemia Foundation was founded in 1954 in Brooklyn, NY, to provide a regular supply of blood donations for local children with thalassemia. It has grown into a large National advocacy group of over 1500 thalassemia patients, and it was a founding member of Thalassaemia International Federation, but its neighborhood focus has been maintained through its network of local chapters across the United States.

How did you come to be involved with CAF?

I joined Cooley's Anemia Foundation twenty-three years ago as National Communications Director after seeing an advertisement in the New York Times. The last 9 years have been spent as National Executive Director.

What are you most proud of since you've been at Cooley's?

We've recognized that patient involvement is really important in making sure we focus on what's important to the community and to represent the diversity of thalassemia. So, over the past decade there's been increasing involvement at every level. We've increased outreach efforts to alpha- thalassemia patients, we've expanded the range and languages of our educational content, and of course Ralph Colasanti became the first thalassemia patient to be elected National President three years ago.

Our long-term collaborative agreement with the US Centers for Disease Control and Prevention (CDC) helped fuel this evolution – we've worked with CDC for over 20 years to expand outreach to under-served thalassemia populations in the United States, and over this time our membership has more than doubled to around 1500 patients.

Other significant achievements include starting our annual Patient and Family Conferences to provide medical updates directly to the patient community and to foster connections among families; additionally, I'm proud of our outreach to a broader network of treatment centers outside of the major thalassemia hubs.

What are the current priorities for CAF?

We are focussed on continuing to improve awareness and understanding of thalassemia among the patient and caregiver communities, to improve access to good quality care for patients, and to support patients and their families on a day-to-day basis. We actively advocate on behalf of patients with the US Food and Drug Administration (FDA) to make sure the patients' voice is heard when they consider new therapies, and we are also actively advocating for a larger pool of adult benign hematologists to be available to patients.

Current new initiatives include:

- Education bursaries for patients and their children.

- Travel support for patients and caregivers to attend their annual treatment center review.

- Health maintenance award for patients who have reached the major milestone of being 50 years or older.

In terms of the future, what are you excited about?

I'm really excited about the new treatment options that are coming – these offer the potential to reduce transfusions, improve quality of life, and even offer the prospect of becoming thalassemia-free. If I had one message to give to patients and their families, it's that thalassemia today is nothing like it was 70 years ago when Cooley's was founded, and it will continue to change for the better over the next 10, 15, and 20 years.

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COMMUNITY RESOURCES

Discover new resources for thalassemia healthcare providers, patients, and their caregivers

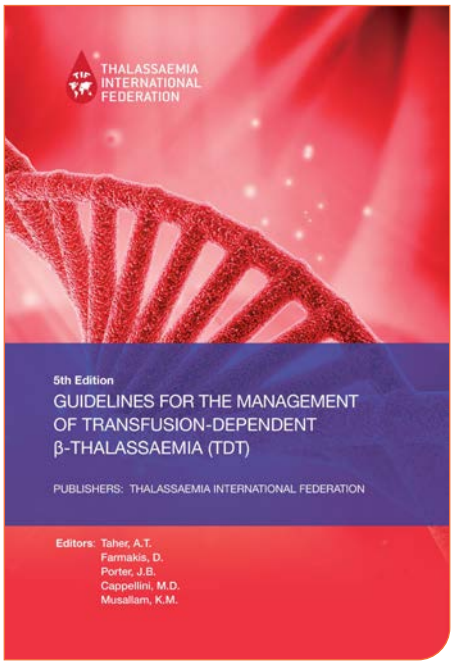
Thal Pals Podcasts

The thalassemia podcasts *Thal Pals: The Alpha Beta Revolution™* aims to facilitate ongoing collaboration between patients, caregivers, and medical experts. The monthly broadcasts feature members of the thalassemia community from around the world discussing currents topics relevant to both α- and β-thalassemia.

Twenty episodes are available and can be accessed here:

Thalassaemia International Federation (TIF) Guidelines

2025 Guidelines for the Management of Transfusion-Dependent β-Thalassaemia (TDT) 5TH Edition have recently been published by the International Thalassaemia Federation. Edited by Dr. Khaled M Musallam, Dr. Ali T Taher, et al., these include contributions from 56 renowned experts in thalassemia from across the world. The updated Guidelines have reorganized content to improve the accessibility of information, while incorporating the latest scientific and clinical developments in understanding the disease, and managing and treating patients with transfusion-dependent β-thalassemia. Content across the 17 chapters spans every aspect of β-thalassemia from its genetic basis through diagnosis, transfusion, iron overload, complications and comorbidities, psychological support, quality of life, multi-disciplinary care, the value of patient engagement, and therapeutic approaches including stem-cell transplantation, gene manipulation, and novel disease-modifying therapies. A useful summary table of monitoring recommendations, and details of TIF's educational programs, are included towards the back of the Guidelines.



The guidelines can be accessed at the TIF website here:

Fast Facts

A series of educational booklets and information sheets written by experts for patients, families, and healthcare providers on the key topics of thalassemia screening, α-thalassemia and, β-thalassemia.

Available in the following languages:

Thalassemia Syndromes: English, Spanish, Italian, Arabic, and French (NEW)
Alpha-thalassemia: Spanish (NEW), Italian (NEW), Thai, Bengali (NEW), Vietnamese (NEW), Urdu (NEW) and French (NEW)
Beta-thalassemia: Spanish, Italian, Vietnamese (NEW), Hindi (NEW), Urdu (NEW), French (NEW), and Bengali (NEW)

You can access and download the Fast Facts resources here:

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CLINICAL RESEARCH UPDATE

Sharing the latest news on clinical research in thalassemia: The Adelphi Thalassemia Disease Specific Programme (DSP)*

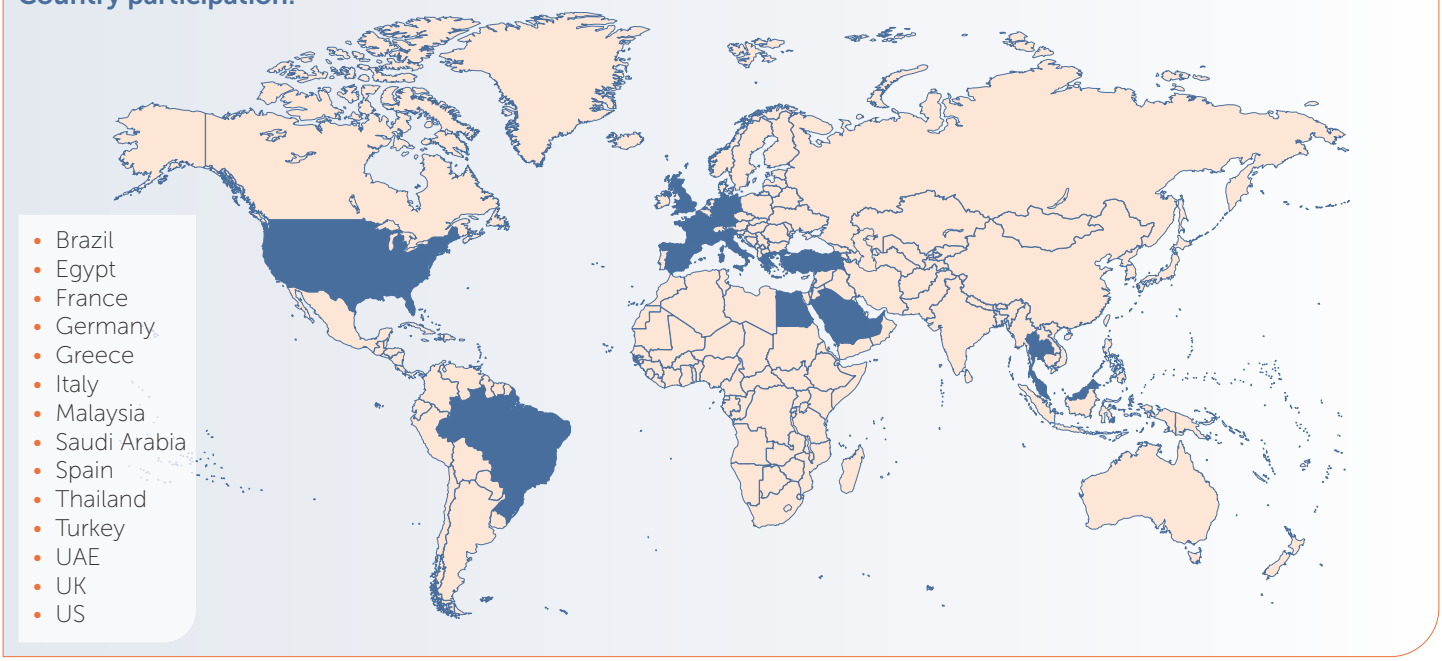
The overall objective of this survey is to capture real-world data to understand current standard of care, patient management, symptomatology, impact of thalassemia on healthcare systems and health-related quality of life, and overall unmet needs for patients with α- and β-thalassemia.

The Adelphi Thalassemia Disease Specific Programme™ (DSP) is an international point-in-time survey of thalassemia-treating physicians and the patients they see. Physicians complete a structured online survey for consecutive patients with α- or β-thalassemia seen during the survey period. The survey includes data on demographics, clinical characteristics, comorbidities, symptoms/complications, treatment, and healthcare resource utilization. Patients are also invited to complete a patient-reported survey while they are in the doctor's office. Patient-reported outcome assessments such as the Functional Assessment of Chronic Illness Therapy – Fatigue Scale (FACIT-Fatigue) and the Work Productivity and Activity Impairment (WPAI), and other questions about health-related quality of life, are included[23].

Inclusion criteria are as follows:

Physician inclusion criteria	Patient inclusion criteria
• Hematologist or hematologist-oncologist • Currently involved in the treatment of patients with thalassemia • Currently manages at least one patient with α- or β-thalassemia • Consented to participate in the study	• Not currently participating in a mitapivat clinical trial • Diagnosed with α- or β-thalassemia • Adult (ages 18+)

Country participation:



The study started recruiting in February 2024 and enrolment completed in Q3 2024.
The data are currently being analysed and results will be shared in due course.
Health Care Providers interested in participation can contact Emily King
emily.king@adelphigroup.com

The Adelphi Thalassemia Disease Specific Programme is owned by Adelphi

EDITORIAL POLICIES & TEAM

The objective of this newsletter is to provide updates on new scientific information, resources, and activities of interest to the thalassemia medical and patient community. The newsletter content is prepared by thalassemia experts in collaboration with Agios Pharmaceuticals. All of these experts serve as paid consultants for Agios Pharmaceuticals.

The following experts are involved in this initiative

- Khaled Musallam, MD, PhD
- Sujit Sheth, MD
- Thomas Coates, MD
- Vip Viprakasit, MD, DPhil
- Ali Taher, MD, PhD
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