WINTER 2025

LIFELINE

Cooley's Anemia Foundation
Leading the Fight Against Thalassemia

Lifeline is a publication of the Cooley's Anemia Foundation • 330 Seventh Ave #201, New York, NY 10001 • **www.thalassemia.org**

Letter from the CAF National President, Ralph Colasanti

Dear Friends,

As mentioned elsewhere in this issue, we are making a big push for donations in our holiday appeal this year. There is so much that we want and need to accomplish and we will be so much better able to do so with an increase in donations. This is especially true because the cooperative agreement grant which we have received from the CDC for more than 20 years has not been renewed at this time. While we hope that it will be put in place at some point in the future, this leaves us with a shortfall that we need to make up. We urgently encourage you to join us in our campaign by making a personal donation and by asking friends, family, and colleagues to lend their support as well. And doing so before 12/31/2025 may be beneficial to you as well. (See page 12)

We also encourage you to contact your elected officials to let them know support of thalassemia is important. (See below for more information.)

This is an exciting and busy time for CAF and for the thalassemia community as a whole. We were thrilled to be a part of the Thalassaemia International Federation's Conference for the Americas in DC in July and thank all of our friends at TIF and the international community for the excellent program. Special thanks, of course, to TIF's Dr. Androulla Eleftheriou, Dr. Panos Englezos, Rawad Merhi, and Lily Cannon, and to CAF's Maria Hadjidemetriou, for all of their hard work and dedication in making this happen.

I was very privileged to be part of the National Organization for Rare Diseases (NORD)'s 2025 Congress and to get to meet so many people representing other rare diseases. In addition, I once again participated in the Sickle Cell Association of America's annual conference. These efforts help CAF to connect with other members of the rare disease community and to find areas where we can work together for our common good. Plus, it's a great opportunity just to meet some truly wonderful and inspiring people.

In addition, CAF has been busy meeting with the offices of numerous Congresspeople and Senators, as well as members of the National Institutes of Health (NIH), to make sure that they know of the needs and interests of all of us with thalassemia. We do everything we can to make our voice heard in a way that matters.

It is an honor for me to be able to represent the thalassemia community as National President of CAF. I am enormously proud of all that we accomplish and once again remind you again that we can accomplish even more with your support.

Thank you.

Sincerely,
Ralph Colasanti

CAF Unveiled New & Improved Website—Thalassemia.org

At the start of the year, CAF proudly launched its redesigned website at thalassemia.org, providing a refreshed digital experience that better supports the thalassemia community, their families, and the medical professionals who care for them.

Powered by Morweb, a nonprofit software and creative agency, the new Thalassemia.org reflects CAF's longstanding mission—to ensure that every individual living with thalassemia lives a longer, fuller life—and renews our commitment to innovation in patient care, education, advocacy, and research.

What's New & Improved:

- A more intuitive, mobile-friendly layout making it easier for patients, caregivers, and clinicians to access resources and information.
- Enhanced "Who We Are" and "What We Do" sections underscoring CAF's 70-plus-year legacy of hope, research funding, patient programs, and educational outreach.
- Clear pathways tailored for key audiences: patients living with thalassemia, medical professionals, family members, and supporters.
- Fresh content and up-to-date materials including:
  - » Treatment and care resources, such as transfusion, iron-chelation, gene-therapy and bone-marrow transplant guidance
  - » Emotional, lifestyle and community support tools geared toward living fully with thalassemia across the lifespan.
  - » Opportunities for involvement: from research fellowships to patient incentive awards and fundraising efforts.
  - » Milestones & Miracles page featuring a timeline of the foundation's efforts in continuing the fight against Thalassemia.
  - » Social Media Post Showcase with real time posting updates.
  - » In depth patient storytelling.

In today's digital era, timely access to trusted information and peer-community connection are critical. By redesigning thalassemia.org, CAF ensures that anyone impacted by thalassemia—whether newly diagnosed, long-time patient, caregiver, or clinician—can find the tools, knowledge and community they need easily. The new site is also optimized to reflect CAF's evolving role in advancing research and supporting life-long care.

Visit thalassemia.org to explore the new site, sign up for the newsletter, and stay informed about upcoming events, funding opportunities, patient-education programs, and ways to help support the work. Share the site with your network—patients, caregivers, clinicians or anyone newly learning about thalassemia.

CAF will continue to update and expand the website's offers, adding more multimedia resources, interactive tools, patient-voices and community features. Launching at the beginning of the year sets the tone for a year of renewed connection, education and hope.

We'd like to send special thanks to our website sponsors Genetix Biotherapeutics, Agios Pharmaceuticals, and Chiesi Global Rare Diseases! We would also like to thank our CAF creative team for their endless work on the creation of the site: Paris Booker (CAF Marketing and Communications Manager), Laurie Pizzo (CAF Board Member), Frank Gazzone, Bob Zeltmann, and Nikhil Vaish.

Let Government Officials Know Thalassemia Is Important

Our elected representatives need to know that their constituents care about thalassemia. We encourage all of you to call your congressperson and senators and let them know that you support funding of programs which benefit people with thalassemia and thalassemia research. (The phone numbers for the House and Senate switchboard is 202-224-3121 as the operator to transfer you to your Senator(s) or your Representative. In most cases you will need to make three separate calls.)

"Hi there, my name is _____ I am a constituent and someone that cares about thalassemia. Thalassemia is important to me because _____. (be very brief) I would like the Senator/Representative to support funding of programs that will benefit people with thalassemia at the CDC and thalassemia research at the NIH in the FY26 Labor Health and Human Services appropriations bill. Thank you for your consideration."

Go Green! Email info@thalassemia.org to receive future issues of Lifeline via email.

Thalassemia: It's in Our Blood Podcast

It's in Our Blood is a new podcast hosted by Laurice Levine & Kathy Raufi. This podcast is created for the entire Thalassemia community including patients, siblings, significant others, friends, healthcare providers, and anyone impacted by or interested in learning more about the genetic blood disorder. Their platform is one where people living with thalassemia and those supporting them can share their personal testimonials, successes, challenges, and questions.

Each episode features authentic voices from patients, caregivers, family members/spouses, providers and others whose lives are touched by Thalassemia. As of Oct. 22, there are a total of 5 episodes live on Apple Podcast and Spotify!

If you would like to be a guest speaker, please reach out to **itsinourbloodpodcast@gmail.com**!

1st Pan American Conference: Thalassemia and Other Haemoglbin Disorders & 2nd Rain Summit

From July 11–13, 2025, healthcare professionals, scientists, patients, and advocates from across the Americas gathered at the Hyatt Regency Crystal City in Washington, D.C. for the 1st Pan-American Conference on Thalassemia and Other Hemoglobin Disorders, held jointly with the 2nd Rare Anemia International Network (RAIN) Summit.

Organized by the Thalassaemia International Federation (TIF) and co-hosted by CAF, Thalassemia Foundation of Canada, the Brazilian Thalassemia Association (Abrasta), and TIF's Pan American Network for Hemoglobin Disorders, this landmark event united healthcare professionals, researchers, policymakers, caregivers, and patient advocates to advance treatment, care, and policy for hemoglobin disorders across the Americas.

The conference opened with remarks from **TIF President Dr. Panos Englezos, CAF National President Ralph Colasanti,** and **Dr. Narla Mohandas**. Their message was clear: collaboration across borders is vital to improving patient outcomes and ensuring equitable access to care.

In his keynote address, **Professor Dimitrios Farmakis** highlighted the importance of regional networks and the growing disease burden of hemoglobin disorders in the Americas. The session **"Patient Voices – My Truth, My Story"** brought the heart of the community to the stage, with inspiring personal testimonials from individuals in the **U.S., Canada, Brazil, Argentina, and Trinidad & Tobago.**

Over the course of three days, participants attended dual tracks designed for both **scientific** and **patient audiences**.

The **Scientific Program** focused on advancing standards of care, exploring new treatments, and sharing emerging research.

Highlights included sessions on:

- **Iron Overload Management & Transfusion Care** (Dr. Thomas Coates, Dr. Paul Telfer)
- **Systemic Complications and Cardiac Health in Thalassemia** (Dr. Maria Domenica Cappellini, Dr. John Wood)
- **Gene and Cell-Based Therapies and the future of curative treatment** (Dr. Stefano Rivella, Dr. David Jacobsohn, Dr. Kevin Kuo)
- **National strategies and newborn screening programs** for early diagnosis and prevention
- **Artificial Intelligence** and Digital Health applications to improve care accessibility

Meanwhile, the **Patient and Parent Program** emphasized emotional well-being, empowerment, and community connection. Sessions led by **CAF's Craig Butler, Robert Ficarra, and Maria Hadjidemetriou** explored coping strategies, mental health, transitioning from pediatric to adult care, and thriving with thalassemia at every life stage.

Workshops addressed **nutrition, exercise, travel safety, family planning, and aging,** while a special **"Meet the Experts"** session gave patients direct access to leading physicians for open discussion and questions.

Uniting for Rare Anemias

The concluding **RAIN Summit** brought together representatives from organizations around the world—including the UK, Brazil, and Trinidad & Tobago—to exchange best practices for improving diagnosis, access to treatment, and patient education for rare anemias.

Experts such as **Dr. Hanny Al-Samkari, Dr. Narla Mohandas, and Dr. Rachael Grace** led sessions that reaffirmed the value of international cooperation in driving sustainable progress.

Support For Travel To Treatment Centers

The Cooley's Anemia Foundation (CAF) is offering support for significant travel to treatment centers for Thalassemia patients. CAF offers limited financial assistance to individuals with Thalassemia who experience financial hardship due to travel expenses incurred in obtaining an annual comprehensive care evaluation or extraordinary Thalassemia care at a major treatment center. (Extraordinary Thalassemia care shall be defined as care recommended by a qualified Thalassemia physician which falls outside regular transfusions or comprehensive care – for example, if a patient requires multiple MRI iron assessments which cannot be performed at their home treatment facility.)

CAF will reimburse up to $500 in travel expenses to qualified applicants. Reimbursements will be distributed on a first come, first serve basis until funding for the period has been exhausted. No one Thalassemia Patient may receive more than $500 travel reimbursement on an annual basis under the terms of this program. Families with more than one child with Thalassemia may be reimbursed for up to $500 for each child. Travel must have occurred on or after July 1, 2025.

Patients are encouraged to take advantage of any existing services already in place for defraying costs (such as low-cost housing or reduced-cost or free travel services provided in conjunction with a specific center) before applying for these funds. Because funds are limited, patients are encouraged to check with CAF in advance if they are planning on utilizing this program for reimbursement. CAF will make every effort to assist qualified applicants but cannot provide funding once it is exhausted for the year. Total funding for the initial year is $30,000.

Requirements:

- Applicant (or their child) must be diagnosed with one of the following forms of Thalassemia: Alpha Thalassemia Major, Beta Thalassemia Major, Beta Thalassemia Intermedia, E Beta Thalassemia, Hemoglobin H disease, or Hemoglobin H Constant Spring.
- Applicant (or their child) must be registered with the Cooley's Anemia Foundation. (If you are unsure if you are registered, please contact CAF at **escott@thalassemia.org**).
- Applicant must be a resident of the United States.
- Applicant must submit copies of receipts for relevant travel-related expenses incurred in order to obtain a comprehensive care evaluation or extraordinary Thalassemia care from a treatment center. See next page for discussion of relevant expenses. Applicant must also submit signed note from doctor or nurse confirming they were seen on the dates for which they are requesting travel reimbursement
- The complete application and receipts for relevant expenses should be returned to **escott@thalassemia.org** or faxed to (212) 279-5999 or mailed to:
  Cooley's Anemia Foundation, Treatment Travel Application, 330 Seventh Ave #201, New York, NY 10001.

Relevant Expenses:

The following are considered relevant travel expenses and can be reimbursed (up to $500) upon submission of receipts:

- Transportation from home to treatment center and back for adult patient or for pediatric patient and one parent/guardian.
- Reimbursement of automobile travel, based upon mileage and utilizing IRS standard mileage rates for medical purposes (21 cents per mile for 2024). Mileage to be determined using distance from home to center and back.
- Basic overnight lodging costs (i.e., cost of room and applicable taxes) for adult patient or for pediatric patient and one parent/guardian. Extra costs (telephone, movie rental, etc.) will not be reimbursed.
- Reasonable meal expenditures for adult patient or for pediatric patient and one parent/guardian. Reimbursable meal costs may not exceed $60 total for one adult patient for one 24-hour period or $120 total for a pediatric patient and one parent/guardian for one 24-hour period.

Aliya Rinaldi: Celebrating 10 Years Transfusion-Free

One inspiring individual from the Cooley's Anemia Foundation (CAF) community is turning a personal milestone into a powerful fundraising movement. On October 21, 2025, Aliya Rinaldi, who was diagnosed in infancy with beta thalassemia, celebrated **10 years since undergoing gene therapy**, and by February 10, 2026, they will mark **10 years without a blood transfusion**.

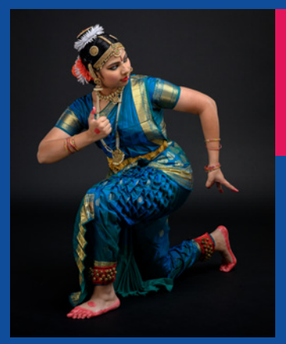
Their journey had decades of transfusion-dependence — and now they're using their story to bring hope to many more.

"As someone who was diagnosed with Beta Thalassemia as a baby and lived three decades dependent on regular transfusions, reaching this milestone is nothing short of miraculous. For the last decade, I have been completely transfusion-free — something I never imagined would be possible.

Ten years of living life on my time instead of a transfusion schedule. Ten years of birthdays, travels, work, friendships, and ordinary moments I never take for granted.

I'm endlessly grateful — to my parents and family, to my husband, to science, to my amazing friends, to my care team, and to every single person who cheered me on near and far."

Aliya is also currently running a fundraiser to "pay it forward" to her Thal Pal community to help the CAF raise funds to continue vital work to support patients, advance treatments, and ensure more people get the chance that she was given! Follow Aliya on social media to learn more @aliyarinaldi



Shae Ghosh's Bharatnatyam Arangetram (Indian classical dance graduation)

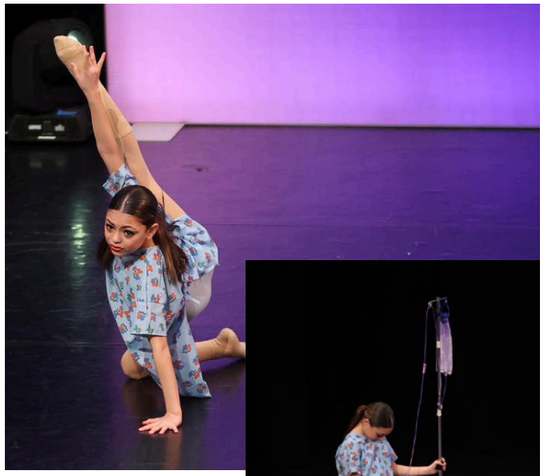
On August 2, 2025, the Cooley's Anemia Foundation had the honor of being part of a truly special occasion — Our Thal pal Shae Ghosh's Bharatanatyam Arangetram, the traditional graduation performance that marks years of discipline and mastery in Indian classical dance.

Shae chose to make her Arangetram even more meaningful by transforming it into a fundraiser for CAF. In lieu of gifts, she invited friends and family to contribute to CAF, raising funds to support medical research, patient outreach, and advocacy efforts.

Her dedication and generosity helped raise well over $3,000, directly benefiting individuals and families affected by thalassemia. Through her performance, Shae not only celebrated a personal milestone but also gave hope to those living with the major form of this genetic blood disorder in the United States.

CAF extends heartfelt congratulations and gratitude to Shae and her family for their incredible commitment and compassion. Her Arangetram stands as a beautiful example of how art, culture, and advocacy can come together to make a lasting difference. We are extremely inspired and honored by Shae's efforts to continue to raise awareness for Thalassemia.

She Danced Through the Pain — and Inspired Everyone in the Room



When 13-year-old **Daniela** stepped onto the stage for her solo dance performance, the audience noticed something unusual. Alongside her sparkling costume and bright smile, she carried an IV pole.

Daniela is a dancer that was diagnosed with **Thalassemia at 14 months old**. She requires blood transfusions every 3 weeks to survive. For 10 years, dance has been her outlet — a place where she could feel strong and free, even when her body felt tired.

Her solo, entitled "***Heal: Her True Story,***" was more than a dance routine. It was a story — her story — about finding strength in the face of pain.

On the day of the competition, she took stage, IV in tow, and moved with grace and courage. Every turn and leap reflected what she lives through every day — the strength it takes to face transfusions, chelation, and the long hours in the hospital.

"It was a very emotional piece, and at some points It was hard for us to get through it. She would be tired; I would be emotional. We really wanted to get the story across, and the point of what she goes through. She competed in it at two dance competitions in a national competition. The Dance is currently blowing up on TikTok and some other social media as well. We hope that it would bring awareness to what she has and that no matter what she has, you could still do the things that you love!"
– Daniela's mom.

Your generosity gives children like Daniela the chance to live, dream, and dance.

Every donation helps fund vital research, patient support, and the hope that one day, no child will have to dance with an IV again.

Donate today at **Thalassemia.org**.

In Memoriam

We regretfully report the loss of patients
Daniel Louie. Rachel Wall, Vatsana Khampong, Samantha Imperiale & Maria Rizzo
and extend our sympathies their friends and families.

Care Walk 2025 Report

This year's Care Walk brought out the very best of our thalassemia community. Across the country, friends, families, and supporters of the Cooley's Anemia Foundation (CAF) came together in an inspiring show of unity, compassion, and hope.

Under the theme "Walk for Your Warrior," participants honored the strength and resilience of individuals living with thalassemia — our true warriors. Together, more than 31 teams joined the effort, raising over $245,000 to support vital programs that make a lasting difference in the lives of patients and families.

From Long Island to Los Angeles, Denver to D.C., and everywhere in between, Care Walk teams gathered in local parks, city paths, and neighborhoods. Walkers carried banners, wore red, and proudly shared who they were walking for — uniting in spirit even when miles apart.

Social media was filled with moments of joy and strength as participants used the hashtag #CAFWalkForWarrior to celebrate their teams and raise awareness nationwide.

Funds raised from Care Walk 2025 directly support CAF's mission to improve and extend the lives of individuals with thalassemia through:

- Research and innovation in treatment and care
- Patient support programs that connect and empower families
- Awareness and education that promote early diagnosis and advocacy

Every step taken, every donation made, and every story shared helps bring us closer to a future free from thalassemia.

CAF extends heartfelt thanks to everyone who participated, donated, and organized local walks this year. Your dedication ensures that no patient walks alone — and that hope continues to move us all forward.

Top 5 teams that raised the most funds this year:

- Westchester/Rockland County organized by Peter Chieco
- Team Ficarra organized by Bob & Marianne Ficarra
- Team Chicago – Blood, Sweat, and Beers organized by Liz Halliday
- Connie's Crew organized by Janice Cenzoprano
- CAF California organized by Maria Saradpon & Alexis Gregorio

We can't wait to see you at Care Walk 2026 as we take even bigger steps together toward a cure!! Stay tuned.

CAF Announces New Fertility/Pregnancy Support Program

The Cooley's Anemia Foundation (CAF) is proud to announce a new program to assist U.S. thalassemia patients with fertility and pregnancy issues. A full application form can be accessed here: **https://bit.ly/CAFFertilityProgram**.

CAF recognizes that individuals with thalassemia may encounter challenges related to fertility and/or pregnancy, often related to thalassemia or its management or to treatments involving bone marrow/stem cell transplantation or gene therapy. In order to provide assistance to meet these challenges, CAF is offering reimbursement of up to $1,000 for approved expenses related to fertility and pregnancy among U.S. thalassemia patients. At this time, this reimbursement may be provided twice during a patient's lifetime for a total of $2,000. An application for reimbursement may be submitted only once per year between July 1 and June 30.

What Expenses are covered?

CAF will provide up to $1,000 in reimbursement for expenses for treatments, devices, activities, and projects which are intended to enable an individual with thalassemia to address fertility and/or pregnancy issues.

Examples of expenses which would be considered for reimbursement (of up to $1,000) include but are not limited to:

- Fertility enhancement processes
- Storage of eggs or sperm
- In vitro fertilization
- Surrogacy-related expenses

Again, these are just some examples to give an idea of the scope of treatments that would be considered.

Applicants must provide proof of expenses paid in order to receive reimbursement. In some instances (such as the use of fertility enhancement processes), CAF may request proof of approval (e.g., letter, prescription, etc.) of treatment, device, activity, etc. by a healthcare provider.

Total expense of treatment, device, activity, etc. may total more than $1,000; however, only a maximum of $1,000 will be reimbursed during any one application period. (For example, receipt may indicate that cost of treatment is $3,000. If approved, CAF will reimburse $1,000 of the $3,000 expense.)

The reimbursement will not be applied to treatments, etc. fully covered by insurance. (That is, if insurance covers all of a treatment, etc., CAF cannot reimburse for that

expense. However, if insurance leaves a portion uncovered, CAF can reimburse up to $1,000 of the uncovered portion.)

CAF will attempt to fund as many applications as possible. Interested individuals should contact Eileen Scott (escott@thalassemia.org) in advance of submitting an application.

Who is eligible?

Any U.S. thalassemia patient who meets all of the following requirements is eligible to apply for support under this program.

- Is registered in CAF's patient database
- Is a resident of the United States and has been a resident for a minimum of 2 years
- Has not already received $1,000 in reimbursement under this program for expenses incurred during the one-year period between July 1 and June 30

How to apply

Applicants should first contact CAF Patient Services Manager Eileen Scott (**escott@thalassemia.org**) to let her know they intend to submit an application. Their email should indicate the amount (estimated if not already expended) to be reimbursed and approximately when they expect to submit their application for reimbursement.

After receiving a response from the Patient Services Manager, individuals should fill out and submit the required application, including any necessary attachments. Attachments include receipts/proof of expenditure; CAF may in some cases request verification that the treatment, device, activity, etc. for which reimbursement is being sought has been approved by an appropriate healthcare provider. Applications may be submitted between July 1 and June 30.

It is CAF's intention to fund all approved requests from eligible patients and will make every effort to do so; however, in the event that requests exceed available funding, CAF reserves the right to regretfully reduce the amount of funds reimbursed to individuals or to deny requests for reimbursement.

CAF Awards $250,000 in Medical Research Fellowships for 2025-2026

We're proud to announce five Cooley's Anemia Foundation Medical Research Fellowships and Grants for the 2025-2026 grant cycle. The total amount of funding for the Research Fellowship Program this year is $250,000.

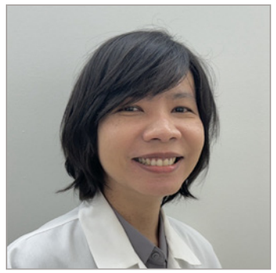
These recipients were assessed on the basis of the quality of the scientific content, the academic accomplishments and future promise of the investigator, the quality of the mentor in the case of postdoctoral fellowships, and, of particular importance, the relevance of the project to the understanding and treatment of Thalassemia.

The CAF Scientific Review Committee reviewed all applications carefully while adhering to the highest standard for scientifically un-biased reviews and made its recommendations for funding to the CAF Board of Directors, who approved those recommendations at its annual Board meeting.

We thank Dr. Janet Kwiatkowski, Chair of the CAF Scientific Review Committee and Medical Advisory Board, and the other members of the Scientific Review Committee for carefully reviewing the grant applications and making these important recommendations. We are excited to see what the investigators learn in these important and cutting-edge experiments.

Meet the Fellowship and Research Grant Recipients:

New Fellowships:



Pinanong Na Phatthalung, PhD

Dr. Phatthalung is a Postdoctoral Fellow in the Ginzburg Laboratory at Icahn School of Medicine at Mount Sinai.

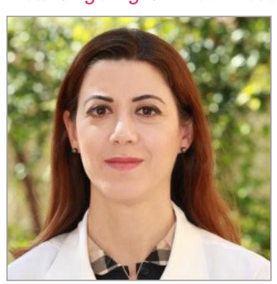
The main goal of the project is to understand the contribution of osteoblastic erythroferrone (ERFE) to the regulation of red cell production in beta-thalassemia. The central regulator of iron balance, hepcidin, is suppressed by expanded red cell production in beta-thalassemia, leading to iron overload even in the absence of transfusion. Specifically, excess bone marrow red blood cell precursors in beta-thalassemia patients and mice produce increased concentrations of another hormone, erythroferrone (ERFE), which leads to suppression of hepcidin. This project aims to increase understanding of how ineffective erythropoiesis is regulated in beta-Thalassemia and provide rationale for targeted development of novel therapeutic approaches for beta-Thalassemia patients.

Maria Eleni Psychogyiou, PhD

Dr Maria Eleni Psychogyiou is a postdoctoral fellow in Dr. Stefano Rivella's lab in the Department of Hematology at the Children's Hospital of Philadelphia.

As a postdoctoral researcher, she is developing innovative gene therapy strategies for α-Thalassemia. Utilizing novel mouse models of the disease, this project will study the underlying pathophysiology and explore potential therapeutic approaches, including lentiviral vectors and in vivo gene editing.

New Ongoing Clinical Research Grant:



Roula Hourani , PhD

Dr. Roula Hourani is a Professor and Chief of the Neuroradiology Division in the Department of Diagnostic Radiology at The American University of Beirut Medical Center.

This research project aims to comprehensively evaluate the neurological and cognitive impact of beta-Thalassemia using advanced brain imaging techniques and standardized neurocognitive testing. The ultimate goal is to establish reliable neuroimaging biomarkers that can guide early diagnosis, monitor neurological progression, and inform clinical decision-making regarding transfusion regimens and preventive strategies for stroke and neurocognitive decline. It is expected to contribute significantly to the understanding of silent neurological complications in beta-Thalassemia.

Renewal Fellowship:

Abhirup Bagchi, PhD

Abhirup Bagchi, PhD is currently a postdoctoral fellow in the laboratories of Dr. Gerd Blobel and Dr. Eugene Khandros, in the Department of Hematology at the Children's Hospital of Philadelphia. Dr. Bagchi completed his doctoral studies on developing lentiviral based gene therapy vectors for hemoglobinopathies at the Centre for Stem Cell Research, Vellore, India.

Dr. Bagchi's research focuses on understanding how individual components of the essential BAF chromatin remodeling complex are involved in regulation of red blood cell production and hemoglobin gene expression. The goal of this project is to identify new therapeutic targets to enhance fetal hemoglobin expression and red blood cell maturation in b-thalassemia.

Ellen B. Fung, PhD RD CCD

Dr. Fung is an Adjunct Professor in the Division of Hematology, Department of Pediatrics within the School of Medicine at the University of California, San Francisco (UCSF). She has a long-standing curiosity on the impact of nutrition and physical activity on bone health in children and adults, particularly those with Thalassemia.

This project is studying if physical activity improve can improve bone health in thalassemia. If effective, this simple, non-invasive, cost-effective, easy to implement exercise therapy could be expanded broadly with minimal effort yet have profound effects.

Anthony Ferrino's Camino De Santiago Walk for Thalassemia

On September 19, Anthony Ferrino of the Cooley's Anemia Foundation's Queens Chapter embarked on a powerful and deeply personal journey—a pilgrimage along the Camino de Santiago in Spain. Anthony has a been with the Queens chapter for over 20 years, constantly showing up for our Thalassemia community.

For centuries, Camino de Santiago has drawn people from around the world seeking reflection, renewal, and purpose. For Anthony, each step became an act of hope and advocacy—a way to raise both awareness and critical funds for those living with thalassemia.

Anthony's journey started on September 19. Carrying with him the spirit of the thalassemia community, Anthony dedicated his trek to every patient, family, and supporter striving for a universal cure. Anthony used TikTok to raise awareness before, during and after his pilgrimage as well. His remarkable effort resonated far beyond the trail, inspiring donors and advocates alike to take part in his mission. Anthony returned from his pilgrimage on September 27.

Thanks to Anthony's dedication, determination and the generosity of his supporters, the pilgrimage raised over $4,000 to benefit Cooley's Anemia Foundation programs—funding vital research, patient support initiatives, and advocacy efforts that bring us closer to a future free from thalassemia.

Anthony's journey is a reminder that every step—whether on a trail in Spain or here at home—can make a difference in the fight against thalassemia.

Urgent: Donate to CAF Before Changes in Tax Law Go Into Effect in 2026

The Big Beautiful Bill (BBB) Act of 2025 introduced changes in the way charitable donations may be deducted starting next year. Starting in year 2026, a floor on deducting contributions has been put into law. The first half of one percent (.5%) of your adjusted gross income (AGI) will not be deductible. Let's say your AGI is $100,000. The first $500 ($100,000 X .5%) of your total contributions for the year will NOT be deductible starting in 2026. This may favor making a contribution by 12/31/25.

The BBB Act also will allow taxpayers who use a standard deduction to deduct up to $1,000 of contributions for single individual filers ($2,000 for married filers) on their tax returns starting in 2026.

Here Are Some Tax Saving Strategies Still Available Under the BBB Act

Let us say you want to make a $10,000 donation to a charity. Instead of sending cash, you can send $10,000 worth of a highly appreciated stock you own. If you take itemized deductions on your individual tax return, you get a deduction for the FULL value of the stock donated. This strategy allows you to avoid a potential capital gain had you sold the stock first and then donated the cash. If you still like the stock, you can buy it back with the cash you were going to use for the donation and now you still own the same stock you had all along but with a higher cost basis.

Another gift giving strategy for individuals who are 73 years and older who are subject to minimum distributions from their IRA, is to make the charitable gifts directly from your IRA to the charity. Any amounts up to $108,000 in 2025 donated to charity from your IRA are excluded from income in calculating your adjusted gross income on your tax return. Under the new tax law, this becomes a huge advantage as there are new potential deductions and tax credits available but get phased out if your income is too high. Donating directly from an IRA up to the $108,000 limit ensures you get the full tax benefit of your gift.

As always, we recommend speaking with your accountant to better understand these tax saving strategies and to see if they are applicable for you.

CAF Incentive Award Programs

CAF is currently accepting applications for our two Incentive Award Programs:

Thalassemia Patient Incentive Awards for 2025-2026

Incentive Awards for Children of Thalassemia Patients for 2025-2026

CAF sponsors incentive awards for U.S. thalassemia patients and for children of U.S. thalassemia patients to further their education and career goals. These awards will be given in March 2026.

Awards offered range from students in a six-month certificate or vocational training program to those pursuing a doctoral degree. Full information about the 2025-2026 Patient Incentive Awards (including eligibility requirements and application forms) can be found at https://bit.ly/PatientIncentiveAwards25; full information about the award for children of patients (including eligibility requirements and application forms) can be found at https://bit.ly/EducationalIncentiveAwards25.

For more information, email escott@thalassemia.org.

Following are lists of individuals who received incentive awards for the 2024-2025 period. CAF congratulates these individuals on their efforts in furthering their education.

Patient Incentive Award winners

The following were among recipients of 2025 CAF Patient Incentive Awards.

- Viraj Amin
- Jiya Bhatt
- Dara Chanthavisay
- Juntra Chanthavisay
- Timothy Charern
- Alexander Cui
- Warner Dixon
- Isabella Gomes
- Lauran Healy
- Rammeet Kaur
- Adrian Ng
- Eleni Paschos
- Nabil F Rahman
- Caitlyn Struven
- Edwin Tan
- Dan Vuong

The following were among recipients of 2025 CAF Incentive Awards for Children of Patients.

- Isabella Abruzzo
- James Abruzzo
- Ryan Colasanti
- Amanda Cosentino
- Alexandra Cosentino
- Emma Marchese
- Kaylin Marchese
- Talitha Martinez
- Andrew Philippou
- Victoria Schevon
- Isabella Shapiro
- Paul Vitaliti

Patient Profile: Shelby Campbell

Before gene therapy, life for **Shelby** was marked by a regular yet demanding routine of blood transfusions and medications. Adopted at the age of two by **Adam and Michelle**, Shelby arrived from China and began her treatment journey at the **Children's Hospital of Philadelphia (CHOP)**, where she received her first transfusion in America.

From then on, Shelby required blood transfusions every three weeks to survive with Thalassemia. To help her body process the excess iron caused by these frequent transfusions, she took **chelation** medication, which removes excess iron from the organs. Her treatment involved a nightly subcutaneous infusion that ran for 12 hours — a small pump attached to her abdomen that slowly released the medication.

Her parents had hoped that the "Sub Q" medicine would reduce her iron overload and allow her a night off each week, but that relief never came. Shelby struggled with not being able to join extracurricular activities, swim, be active, socialize, and enjoy childhood the way other children could. Despite these challenges, Shelby appeared just like any other child — though the effects of Thalassemia were invisible to others.

Over time, Shelby's family created a sense of routine and even fun around transfusion days, transforming them into meaningful, if long, experiences at the hospital filled with love and care.

When Shelby's iron levels remained high, her parents met with the transplant team to explore other options. After careful consideration, they chose a new and uncharted path — **an FDA-approved gene therapy by Bluebird Bio (now known as Genetix Pharmaceuticals)**. This therapy involved a stem cell transplant using Shelby's own stem cells, meaning she was both the donor and the recipient. The journey required a two-month hospital stay followed by seven months of home isolation.

Before hospitalization, Shelby underwent several pre-operative medical visits. Her transfusions were increased from every three weeks to every two weeks to prepare her body for the chemotherapy she would receive prior to the actual gene therapy procedure.

Throughout this period, her mom stayed in the hospital with Shelby day and night, while her dad visited on weekends and her grandparents ("Gram and Papa") came by twice a week. Shelby kept in touch with friends through Facetime playdates and even waved to them through the hospital window. She formed deep, meaningful connections with her nurses and medical team — whom she missed dearly after discharge.

To bring some joy and structure to their hospital days, Michelle organized **"theme days"** with fun focuses like puppies, the ocean, Stitch, unicorns, and science. These special days helped lighten the mood during difficult times as Shelby faced ailments, hair loss, and homesickness. On those days, she would hand out treats to nurses, staff, and other kids in the hospital. To make bath time more enjoyable, her parents created nightly **"spa nights,"** turning ordinary routines into moments of comfort and joy.

By focusing on small moments of fun and connection, Shelby was able to look back on her challenging hospital experience with fond memories.

Michelle shared, "Our family was fortunate to receive tremendous support from the entire CHOP staff and the Bluebird Bio (Genetix Pharmaceuticals) patient care team. Both organizations rallied around us, ensuring our stay was as comfortable and stress-free as possible so that we could focus entirely on supporting Shelby's hospital stay and a successful gene therapy procedure."

Since leaving the hospital, Shelby has not needed a single blood transfusion. She now undergoes monthly **phlebotomy** for chelation, meaning she no longer needs nightly oral or subcutaneous medication. Her energy levels remain stable, and her hemoglobin hovers around 11 — even with the phlebotomy. Shelby's body is now producing healthy hemoglobin on its own.

She continues to connect with peers who have also undergone gene therapy and understand what it's like to live with Thalassemia. Recently, she has been sharing her story with others who are considering gene therapy, taking pride in her bravery both before and after her treatment. She is now monitored only by her bone marrow transplant specialist and hematology teams.

Michelle's advice to other parents and caregivers considering gene therapy for their children with Thalassemia is:

"As a mother, the most important thing is to focus on making memories and having fun — and to ask for help. We wish we had done that more and made better use of educational resources. Don't hesitate to reach out to your community for support. Ask as many questions as possible. We relied heavily on other families going through the same process. I also recommend asking not only about gene therapy itself but also about the care that follows. It's important to know what resources are available and to have realistic expectations for what lies ahead. And always remember to look back and acknowledge how far you've come."

Today, Shelby is **9 years old and 19 months post–gene therapy**. Her parents lovingly call her their **"joy walker."** She loves life and lives it to the fullest! She is active and involved in dance, gymnastics, school, and church. At home, she's often playing with neighborhood friends — doing many of the things she couldn't do before.

Shelby can now live like every other child, with only a few limitations, and her family couldn't be more grateful.

Legacy Society Registration Form

The Cooley's Anemia Legacy Society recognizes those who have made a commitment in their estate plan to provide enduring support for Cooley's Anemia Foundation to future generations. Thank you for choosing us!

As evidence of our/my desire to provide a planned gift in support of Cooley's Anemia Foundation, we/I hereby inform you that we/I have made a provision for a planned gift. We/I understand that this commitment is revocable and can be modified by us/me at any time. Our/my gift has been arranged through a:

- ☐ Last Will and Testament
- ☐ A Codicil in a Will
- ☐ Individual Retirement Account Charitable Rollover
- ☐ Charitable Gift Annuity
- ☐ Gift of Life Insurance
- ☐ Gift of Real Estate
- ☐ Retirement Plan Beneficiary Designation
- ☐ Charitable Remainder Trust
- ☐ Charitable Lead Trust
- ☐ Transfer on Death (TOD) on Account
- ☐ Contribution to Endowment
- ☐ Other _____

We/I have made our/my designation to
Cooley's Anemia Foundation, Tax ID # 11-1971539, 330 Seventh Avenue, Suite 201, New York, NY 10001.

- ☐ We/I would like for my/our planned gift to be:
  - ☐ Unrestricted – for maximum flexibility to meet future needs.
  - ☐ Restricted – please share your intentions with us so we can ensure that they are consistent with the Gift Acceptance Policy of Cooley's Anemia Foundation. Contact us so we can help you with this important decision.
- ☐ Please enroll us/me in the Cooley's Anemia Foundation Legacy Society.
  - ☐ You may publish our/my name(s) as (a) society member(s).
  - ☐ Please consider this to be an anonymous gift.
- ☐ Please send more information about including Cooley's Anemia Foundation in my/our estate plans.

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Address _____

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Signature _____ *Date* _____

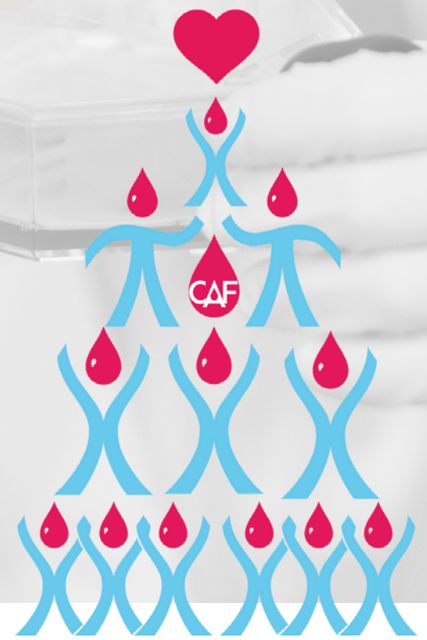
Signature _____ *Date* _____

Please return to:
Cooley's Anemia Foundation, 330 Seventh Avenue, Suite 201, New York, NY 10001

Telephone: (212) 279-8090, ext.201
Email: info@thalassemia.org

This holiday season, your gift brings *hope* to patients and families fighting thalassemia.

Learn more at thalassemia.org/donate.



$100 – Supports patients learning from each other through CAF social media

$250 – Educates newly diagnosed patients and families

$500 – Covers testing costs for early heart and liver damage detection

$1,000 – Helps one patient pursue the dream of having a child

$2,000 – Provides travel and hotel for a family of four to attend the Patient-Family Conference

$5,000 – Sends 10 patients to specialized thalassemia clinics

$7,500 – Helps 5 patients pursue a college education

$10,000 – Funds vital medical research for future breakthroughs

Cooley's Anemia Foundation
70 Years of Leading the Fight Against Thalassemia

Tax changes coming next year may mean some tax filers could benefit from making charitable gifts this year.

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