WINTER 2024

LIFELINE

COOLEY'S ANEMIA FOUNDATION

LEADING THE FIGHT AGAINST THALASSEMIA

Lifeline is a publication of the Cooley's Anemia Foundation • 330 Seventh Ave #200, New York, NY 10001 • **www.thalassemia.org**

LETTER FROM THE CAF NATIONAL PRESIDENT, RALPH COLASANTI

Dear Friends,

CAF celebrated its 70th anniversary in 2024, and it's a tremendous opportunity to take a look back at how things have changed for all of us in the thalassemia community.

Back in 1954, doctors were still struggling to figure out the best way to treat thalassemia. They knew that transfusions were needed, but it took a lot of time to figure out when and how much to transfuse. This was especially problematic because there were no iron chelators at the time, so they had to figure out how to balance the need for blood to treat the anemia with the iron overload that would build up. As a result, patients' lives were so much shorter than they are now. Most were fortunate if they lived into their 20s. And there was so much more we didn't know about how to treat the many complications that were common with thalassemia.

Today, it's a whole new ball game. While every patient is different and requires specialized care, doctors now have so much more knowledge and tools to use when figuring out appropriate transfusion and chelation schedules. Managing thalassemia and its complications is still a big challenge, but individuals who get treatment from knowledgeable doctors are living longer and thriving. We are going to college, having rewarding careers, raising families (and even becoming grandparents!), and doing so much more that those in the past could only dream of. And some of us are even benefitting from curative gene therapy and bone marrow transplant options!

This all comes about because of the dedication and work of so many people. The clinicians and researchers who have striven to understand how thalassemia works and what can be done to address its challenges. The patients and families who have shared their time and experiences in clinical trials, surveys, studies, and other activities that have added to that fund of knowledge. The foundations, corporations, government agencies, and generous individuals who have provided the support for that knowledge. The untold number of people who selflessly donate blood so that people like me can live. All those who donate financially to CAF, as that generosity is our lifeblood. And of course the many incredible, devoted people who have been a part of CAF on so many different levels, as Board officers and members, chapter members, volunteers, donors, allies, and staff.

Thalassemia in 2024 is unrecognizable from thalassemia in 1954. Thank all of you and every single person who has made that so. We are blessed to have come so far and to have had so many people thinking of us, caring about us, and working to make our lives better.

With gratitude,
Ralph Colasanti

Partial funding for this issue of Lifeline is provided by Agios Pharmaceuticals and bluebird bio.

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A NEW USER-FRIENDLY EXPERIENCE FOR PATIENTS, DONORS AND DOCTORS

CAF is launching their newly redesigned website, offering a fresh, modernized platform to better serve the thalassemia community & potential new donors. Built with user experience at its core, the website now features an intuitive interface, robust resources, proprietary branding, and an inclusive design to empower patients, families, and healthcare providers in their journey to understand and manage this genetic blood disorder.

Streamlined Navigation for Better Access

The revamped site introduces an easy-to-navigate menu system, ensuring visitors can quickly find critical information about thalassemia, including symptoms, treatments, and ongoing research. It is tailored and broken down by the foundation's primary users including: Patients/Caregivers, Medical Professionals and Donors. Users can also locate support programs, scholarships, and patient advocacy resources in just a few clicks.

Heartfelt Storytelling from our Patient Community

Like CAF has demonstrated through social media, the website will also be the hub for the patient community to share their stories. Users will get to read and watch content from a number of CAF's thalassemia warriors on what inspires them to continue to thrive with thalassemia and how you can help support CAF in its efforts to find a universal cure for all patients.

Engaging Call to Actions for Potential New Donors

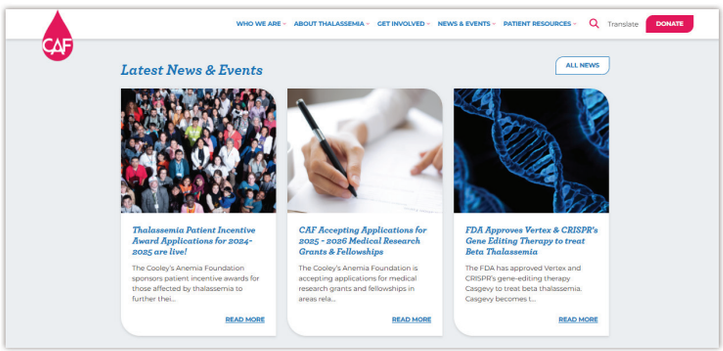
It is important to celebrate all the accomplishments that the foundation has had throughout the years. This website now features a timeline that shares all the major milestones that the foundation has made possible with your support and all the hard work and time put in. You are able the scroll through to see what years of help has done for not only the foundation but for all people with thalassemia.

Highlighting Historical Milestones & Miracles

It is important to celebrate all the accomplishments that the foundation has had throughout the years. This website now features a timeline that shares all the major milestones that the foundation has made possible with your support and all the hard work and time put in. You are able the scroll through to see what years of help has done for not only the foundation but for all people with thalassemia.

A Modern Look with Accessibility in Mind

The site's clean, visually appealing design reflects the foundation's mission of providing clarity and hope. Prioritizing inclusivity, it incorporates accessibility features to ensure individuals with disabilities can navigate and benefit from the content with ease.



A New Era for Cooley's Anemia Foundation

The Cooley's Anemia Foundation's redesigned website stands as a testament to its dedication to improving the lives of those affected by thalassemia. It's more than just a website, it's a home that connects, educates, and supports the global thalassemia community.

Explore the new website in 2025 and discover how this refreshed platform can help you or your loved ones navigate life with thalassemia.

2024 THALASSEMIA PATIENT-FAMILY CONFERENCE

The Thalassemia community came together for the 2024 Patient-Family Conference, a three-day event filled with knowledge-sharing, personal connections, and inspiring moments. Hosted from Friday, July 12th, to Sunday, July 14th, the conference brought together patients, families, medical professionals, and advocates to deepen understanding of Thalassemia and support those living with the condition.

Friday, July 12th: A Day to Begin and Connect

The event started with introductory sessions on Friday evening, including Thalassemia 101 by Jennifer Eile MS, PNP-BC aimed at providing fundamental insights into the condition. Parents had a dedicated meeting to connect, share experiences, and explore ways to support their children. The community came together after for a welcome dinner and words of inspiration by our National Director Craig Butler and our National President Ralph Colasanti.

Saturday, July 13th: A Focus on Advances and Community

Saturday was packed with educational opportunities and engaging activities. Sessions tackled critical topics such as Chelation Challenges, Adrenal Insufficiency in Thalassemia, and Thalassemia & Nutrition. Attendees also gained insights into cutting-edge developments with New & In Development Therapies and Gene Therapy Updates. Special thanks to Jeanne Boudreaux MD, Zahra Pakbaz MD | HS, Ellen Fung PHD, RD, CCD, Amy Tang MD, Radhika Sawh, and Mallika Venkatramani for joining our conference to spread all of this vital information to our community. The evening ended with a lovely Dinner Dance and a fun costume contest to cap the night off! Our adult patients had the pleasure of getting together for an interactive paint & sip after the dinner dance ended.

Sunday, July 14th: Wellness and Reflection

The final day emphasized wellness, community, and creativity. Participants began with yoga, followed by sessions tailored for children, parents, and patients. Unique offerings like the Friends, Siblings, and Spouses Session created space for discussions about how Thalassemia impacts extended relationships. The day concluded with a thought-provoking Bloodstream Media podcast session sponsored by Agios "Thal Pals: The Alpha Beta Revolution." We had the pleasure of having our inspirational thal pals speak about their experiences with thalassemia. Special thanks to Pranav Saha, Robert Mannino, and Dr. Zahra Pakbaz for sharing their stories and leaving attendees inspired and motivated. We also must shoutout Shae Ghosh and Dr. Kevin Kuo for hosting this incredible episode. Make sure you check out Bloodstream Media for more Thal Pals Podcast episodes!

A Community United

The 2024 Thalassemia Patient-Family Conference underscored the importance of education, innovation, and emotional support. It reinforced the power of community in navigating the challenges of Thalassemia and celebrated the strength of those who live with it every day.

We'd like to thank our wonderful sponsors for always helping us bring these events to life. That includes:

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STAYING A PART OF THE COMMUNITY: TALKING WITH JULIA TANG

Julia Tang was born with beta thalassemia but received a bone marrow transplant (BMT) at age 10 and has been transfusion-free ever since. Now 23 years of age, Julia has remained in touch with CAF and a part of the thalassemia community. CAF spoke with Julia recently about her experience and her life since her BMT.

CAF: What do you remember about the whole BMT experience?

Julia: What I remember the most from my experience isn't the chemo, medications, and procedures I underwent to receive my transplant. I remember how well my doctors and nurses treated me, and how my mom slept in my hospital room with me every night. During my actual transplant, I remember the doctors and nurses covered the IV bag with my new bone marrow with sparkles and stickers, and my transplant was just like a transfusion. Although I had to leave school and lost all my hair, my doctors, nurses, and family provided a sense of comfort and normalcy that took away any nerves I had.

I understand that you have a different blood type post-BMT than you had before.

Yes. My blood type changed from O to B after my transplant, which I didn't know was possible until afterwards. Now it makes me even more grateful for and connected to my donor since we now share a bone marrow and we have the same blood.

You have stayed connected to the thalassemia community after being cured. Could you tell me a little about that?

As a child and teenager after I was cured, I realized I could live the rest of my life without limitations of living with thalassemia, but I never wanted to remove myself from the community while so many others are still unable to access the cure I was lucky enough to receive. There is still work to be done to allow all thalassemia patients to access treatment and a cure, and I have found that my own experience has given me a unique perspective that can help others as well.

Serving on the Pan-Asian Committee for CAF has also been extremely fulfilling, as we can focus on reaching Asian American communities to provide connection and support, a mission that has been especially fulfilling for me as I am able to provide meaningful support for communities I hold great personal value in.

What was it like the first time you were able to donate blood?

Growing up with thalassemia, it never crossed my mind that I would ever be able to donate blood. The first time I donated it was at the hospital I work at. They hosted a blood drive that day, and I thought, "Why not?" I realized there was no reason I should no longer be able to donate and have found it fulfilling to be able to donate regularly since.

Could you share a little about speaking to people at blood center events?

I have spoken at events for donors as well as chairpersons responsible for organizing blood drives for the New York Blood Center. While the prospect of being vulnerable enough to tell my personal story with thalassemia and my journey to being cured and able to donate blood myself was daunting at first, it has been a true honor to connect with the community and encourage others to donate blood. This experience has also been special to me as I have been able to show gratitude to the community of people who provided support for patients like myself through blood donations, and I was able to show them how their generosity was the thing that allowed me to be there.

What are your future plans?

My own experience as a patient has guided my values and priorities, and I hope to become a physician in the future. I am currently working as a research associate at Mount Sinai and am also in the application process for medical school and will hopefully attend next Fall.

Is there else you would like to say?

I am so grateful for the CAF community for not only the support and celebration of my experience, but for the strength and effort everyone at CAF puts into supporting patients and striving for a cure that can be accessible to all. I hope that I can continue to do whatever I can to spread awareness about thalassemia, and I hope that anyone that is affected by thalassemia can find support and inspiration within our community.

CAF thanks Julia for sharing her story and for continuing to work on behalf of the thalassemia community.

50+ HEALTH MAINTENANCE AWARD

The Cooley's Anemia Foundation is announcing support for health maintenance in thalassemia patients 50 years & older. We are recognizing the importance of maintaining health among thalassemia patients and is offering reimbursement of up to $500 for approved expenses related to health maintenance among U.S. thalassemia patients who are age 50 or older.

What expenses are covered?

CAF will provide up to $500 in reimbursement for expenses for treatments, devices, activities, and projects which are intended to improve or maintain an individual's health and which have been discussed with and approved by an appropriate treating physician and reimbursement for which has been agreed to by prior arrangement with CAF. The health issue which is being addressed does not have to be related to thalassemia, so long as the goal is improvement or maintenance of overall health or specific aspects of health of an individual.

Examples of expenses which would be considered for reimbursement (of up to $500) include but are not limited to:

- Purchase/rental/use of medical devices approved by healthcare provider to treat medical/health condition (such as low intensity vibration therapy devices aimed at improving bone strength, etc.)
- Cost of vitamins recommended by healthcare provider which are not covered under health insurance plans
- Uncovered co-pays associated with MRI measurements
- Membership in a gym or classes intended to improve physical health such as yoga, etc. (such activities having been approved by healthcare provider)
- Counseling sessions uncovered under health insurance
- Please note that at this time we are unable to reimburse you for the cost of iron chelators and transfusions.

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, as well as proof of approval (e.g., letter, prescription, etc.) of treatment, device, activity, etc. by healthcare provider.

Total expense of treatment, device, activity, etc. may total more than $500; however, only a maximum of $500 will be reimbursed. (For example, receipt may indicate that cost of treatment is $1000. If approved, CAF will reimburse $500 of the $1000 expense.)

Who is eligible?

Any 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
- Has reached an age of 50 years or older
- Is a resident of the United States and has been a resident for a minimum of 2 years
- Has not undergone a successful bone marrow transplant (BMT) or gene therapy procedure more than 5 years ago
  - » Patients who have undergone a successful BMT or gene therapy procedure within the last 5 years are still considered eligible
- Has not already received $500 in reimbursement under this program for expenses incurred during the one-year period between July 1, 2024 and June 30, 2025

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, as well as 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, 2024 and June 30, 2025, for expenses occurred during that period (between July 1, 2024 and June 30, 2025).

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

In Memoriam

We regretfully report the loss of patients

Jacob Hawkins • Ourania Cioffi • Francine Jasunas

and extend our sympathies their friends and families.

CARE WALK 2024 REPORT

The 14th annual Care Walk turned out to be a huge success! This year, CAF was able to successfully pass our Care Walk fundraising goal of $220,000 by raising a total of $245,000.

We'd like to thank all 427 attendees for making this Care Walk one of our most successful walks to date.

The Care Walk holds immense significance as it serves as a powerful celebration of people with thalassemia. Thalassemia is a genetic blood disorder that affects the production of hemoglobin, resulting in anemia. By participating in the Care Walk, you can help raise awareness about thalassemia and support those affected by this condition.

The goal for Care Walk 2025 is to make it bigger than ever before, and this is where you come in. Your involvement and support are crucial in achieving this objective.

You can get involved by registering early for next year's Care Walk and contribute to making it the largest and most impactful event in the history of Care Walks.

Through your participation, you not only join a community of individuals who share a passion for making a difference but also help to create a positive and lasting impact. By spreading the word and encouraging others to join the cause, you can increase the reach and effectiveness of this remarkable event.

If you require any further assistance or have questions, please feel free to reach out to Paris Booker at 212-279-8090 x 208 or pbooker@thalassemia.org. Paris is more than willing to provide you with the necessary information and support to ensure your Care Walk experience is both enjoyable and meaningful.

Let's unite in the spirit of Care Walk 2025 and make a real difference in the lives of those affected by thalassemia. Together, we can create a supportive and empowering community that raises awareness, funds vital research, and brings hope to individuals and families impacted by this condition. Let's all find a universal cure for thalassemia!

Top 5 teams that raised the most funds this year:

- Westchester/Rockland Chapter's Team organized by Peter Chieco
- Team AJ organized by Joe & Lisa Vigliotti
- Chicago CAF organized by Arianna Bonomi &Teresa Tomaino
- Team Zayna organized by Sarah Connolly
- Team Ficarra organized by The Ficarra Family

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CAF EARNS SPOT ON GREAT NONPROFITS TOP RATED 2024 LIST

We're extremely excited to announce that The Cooley's Anemia Foundation was recognized by GreatNonProfits in 2024! The Foundation's hard work and dedication for all thalassemia patients has resulted in GreatNonProfits listing CAF as a Top-Rated non-profit organization in 2024. GreatNonProfits, a non-profit organization that assesses the credibility and impact of all non-profit organizations. They are best known for their "Top Rated" awards, given out to the nonprofit organizations that maintain an average rating of 4.5 stars throughout the entire year. The Cooley's Anemia Foundation is grateful to be awarded this year, and we will continue to carry on our mission to ensure that all thalassemia patients will be cured. We will continue to be a beacon of hope for all those battling thalassemia and for those dealing with their loved ones struggling with the blood disorder. Special thanks again to GreatNonprofits for this great recognition and we hope to be awarded again in 2025!

CAF AWARDS $250,000 IN MEDICAL RESEARCH FELLOWSHIPS FOR 2024-2025

We're proud to announce five new Cooley's Anemia Foundation Medical Research Fellowships for the 2024-2025 grant cycle. The total amount of funding for the research Fellowships is $250,000.

These Fellowship 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.

Dr. Jun Liu, MD., PhD

Dr. Jun Liu is a research fellow under joint mentorship by Dr Daniel Tenen and Dr Li Chai, at Harvard Medical School. She is also a clinical fellow in Transfusion Medicine at the Harvard Combined Transfusion Medicine fellowship program, with clinical and research interests in hemoglobinopathies.

Dr Liu is characterizing a novel small molecule degrader of ZBTB7A/LRF, a transcriptional repressor of gamma globin, as a means of inducing gamma globin expression in human erythroid cells. This approach can offer potential therapeutic benefit in beta-globinopathies such as sickle cell disease and beta-thalassemia.

Dr Liu is working closely with medicinal chemists to further refine the molecular structure of the compound to optimize its potency and minimize its toxicity.

Ellen B. Fung, PhD RD CCD

Dr. Ellen B. 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. She is co-author of the first textbook related to the assessment of pediatric bone health, and in 2023 authored a guidebook on nutrition for patients with Thalassemia, published by the Thalassemia International Federation.

Dr. Fung is the Director of the Bone Density Clinic at UCSF Benioff Children's Hospital Oakland and the Director of the Summer Student Research Program; a UCSF summer research internship for underrepresented students passionate about science. When she is not in the office, or mentoring students, she enjoys swimming, hiking, gardening and baking.

Vanessa Yingling, PhD

Dr. Vanessa Yingling is a Professor of Kinesiology at California State University, East Bay. She is trained as a biomechanist with specific training during a post-doctoral fellowship in bone mechanics and physiology. As a PI on NIH-funded grants, she has experience using animal models to investigate factors that affect bone strength development.

In 2013 she transitioned her research to focus exclusively on human subjects. She has contributed to science establishing the relationship between field tests of muscle function and bone strength in athlete and healthy populations. She has worked with American Bone Health in the revision the NIH Best Bones Forever program into a broad-based curriculum for both girls and boys that can be implemented in after school programs and camps and currently serves on the Fitness Gram Advisory Board.

Most recently, Drs. Yingling and Fung collaborated on a project to understand bone strength relative to body size in patients with thalassemia, which led to the current exercise intervention project.

A short description of the project

Over half of all adults with thalassemia (Thal) have low bone mass and are at significant risk for fracture. The reasons for low bone mass are multifactorial, though bone morbidity is due, in part, to inactivity. Bisphosphonate medications often used to treat osteoporosis have many side effects and are often contraindicated in patients with thalassemia. Therefore, non-pharmacological strategies are needed to not only improve bone health and reduce fracture risk, but may also alleviate bone pain.

In non-Thal populations, physical activity is known to increase muscle mass, improve bone density and strength, and reduce pain. Patients with Thal are less active compared to their peers, and we have shown bone deficits are associated with inactivity. Previous studies suggest that lifestyle factors can influence bone health in thalassemia. The logical question remains, can physical activity improve bone health? This study is the natural, most plausible, progression to correcting the significant burden of osteoporosis in thalassemia.

In year one of this study, we propose a 3-month home based exercise intervention. The short-term outcomes to be explored are improvements in muscle mass and function- precursors to long term bone outcomes as well as pain and quality of life.

We plan to extend the intervention with a second year of funding, and direct bone outcomes will be assessed. If effective, this simple, non-invasive, cost-effective, easy to implement exercise therapy could be expanded broadly with minimal effort yet have profound effects.

Audrey Belot, PhD, Post Doc Fellow

The objectives of my Dr. Belot's PhD were to study iron metabolism regulation and to identify new therapeutic strategies for diseases linked to iron metabolism. Using an IRIDA mouse model, Dr. Belot showed that a hepcidin-suppressing molecule corrects the iron deficiency and anemia in IRIDA. She was also interested in the role of iron in non-alcoholic fatty liver disease (NAFLD), which can progress in some cases to a more severe stage, non-alcoholic steatohepatitis (NASH), for which no treatments are available.

Dr. Belot showed that hepcidin production is increased in this disease resulting in liver iron retention, which aggravates NAFLD. The characterization of this dysregulation has led to identifying a promising new therapeutic target, HuR, to prevent disease progression. Her third project focused on regulating hepcidin expression through an anti-matriptase-2 antibody in β-thalassemic mice to improve anemia and iron overload.

As a post-doc, Dr. Belot joined Dr. Hamza's lab to study heme metabolism which is highly complementary to iron metabolism as the majority of iron is within heme. For her postdoctoral research, Dr. Belot specifically addressed the role of the heme transporter HRG1 in red cell maturation in both steady-state and disease conditions (Sickle cell disease, β-thalassemia).

This project combines her experience studying red cell development during her Ph.D. with heme homeostasis and transport during her Postdoctoral research. Dr. Belot's short-term goal is to have a better understanding of the interplay of red blood cells metabolism and heme at steady-state and during diseases such as β-thalassemia to find innovative therapeutic strategies for those patients.

Her long-term goal is to establish her own laboratory and lead a research group as a PI on the implication of the unappreciated trafficking of heme between cells in different organs and its role in both steady-state and disease conditions.

Abhirup Bagchi, PhD

Dr. Bagchi 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.

Dr. Shobana Navaneethabalakrishnan

Dr. Navaneethabalakrishman is a biotechnologist with over a decade of expertise in reproductive biology. She obtained my master's degree in biotechnology from Bharathiar University, India, where her research focused on the impact of titanium dioxide nanoparticles on reproductive organs of female mice.

As a Junior Research Fellow at the University of Madras, Dr. Navaneethabalakrishman investigated the effect of hexavalent chromium on the hypothalamo-hypophyseal-gonadal axis. Her doctoral studies at the University of Madras centered on understanding the molecular mechanisms underlying the effect of gestational exposure to hexavalent chromium on Sertoli cells in F1 progeny.

At Texas A&M University, as a postdoctoral researcher, she explored the intersection of hypertension and reproductive biology, specifically studying the role of gonadal macrophages in hypertension-associated reproductive dysfunction. To further deepen her knowledge of macrophage biology and its implications in disease models, Dr. Navaneethabalakrishman joined Dr. Francesca Vinchi's lab at New York Blood Center, where she developed a keen interest in investigating the role of iron/heme-driven macrophages in the pathogenesis of beta-thalassemia.

The primary focus of the current study is to elucidate the role of heme/iron-activated macrophages in bone marrow (BM) dysfunction in beta-thalassemia, focusing on their impact on ineffective erythropoiesis and regulation of hematopoietic stem/progenitor cell (HSPC).

Furthermore, she will also evaluate therapeutic strategies aimed at modulating macrophage function and heme scavenging to alleviate BM inflammation, improve erythropoiesis, and preserve HSPCs. This research endeavors to provide insights into novel treatment approaches for β-thalassemia, potentially leading to enhanced clinical outcomes for affected individuals.

SUPPORT FOR TRAVEL TO TREATMENT CENTERS FOR THALASSEMIA PATIENTS

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, 2024.

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 #200, 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.

CAF PATIENT SOCIAL GROUPS

Each Sunday in the beginning of the month, our patient social worker Kathleen Durst hosts a virtual thalassemia patient support group! These inclusive virtual groups are designed for thalassemia patients of all ages to meet with one another to discuss all things mind wellness in the thalassemia community, including tips and techniques to increase mind and emotional wellness!

The patients break out into separate zoom sessions with one another depending on their age range. You can join these groups by signing up on thalassemia.org or by reaching out to our communications manager Paris Booker at 212-279-8090 x 208 or pbooker@thalassemia.org. Kathleen Durst continues to work hard in the name of mind wellness for patients that need inspiration, motivation, and positivity. Her work here at CAF never goes unnoticed and the CAF team is extremely proud to have her continue this work!

AGIOS DRUG REDUCES BLOOD TRANSFUSIONS IN PATIENTS WITH BETA-THALASSEMIA

Agios Pharmaceuticals announced in June that its drug Pyrukynd (mitapivat), an oral pyruvate kinase activator, reached its primary endpoint in a Phase III trial evaluating adults with the blood disorder transfusion-dependent alpha- or beta-thalassemia.

The Phase III ENERGIZE-T trial's primary endpoint was transfusion reduction, defined as a 50% or lower reduction in transfused red blood cell units. Treatment with the drug was statistically significant compared to placebo, as the Pyrukynd arm showed 30.4% of patients reached a transfusion reduction response compared to just 12.6% in the placebo group, with a p-value of 0.0003.

A total of 258 adult participants with transfusion-dependent (TD) alpha- or beta-thalassemia were enrolled in the ENERGIZE-T study. These patients were randomized into two treatment arms — 171 patients who received a 100mg oral dose of Pyrukynd and the remaining 87 patients who were administered placebo — over a 48-week treatment period.

A higher number of patients in the Pyrukynd arm also reached the secondary endpoint of transfusion independence, defined as being transfusion-free for eight or more consecutive weeks. Patients on the drug also showed a statistically significant reduction in additional measures of transfusion reduction response. Adverse events were similar across both Pyrukynd and placebo groups.

Agios plans to present a more detailed analysis of ENERGIZE-T's data at an unspecified medical meeting.

The trial's results come as Pyrukynd reached its primary endpoint of hemoglobin response in a Phase III trial in patients with non-transfusion-dependent alpha- or beta-thalassemia in January 2024. In that trial, 42.3% of patients in the Pyrukynd arm netted a response versus just 1.6% in the placebo arm.

"Building on the compelling data generated in the Phase III ENERGIZE study of mitapivat in adults with non-transfusion-dependent alpha- or beta-thalassemia announced earlier this year, today's results underscore the potential of mitapivat, with its unique mechanism of action improving red blood cell health, to be a meaningful oral treatment option for all thalassemia patients, regardless of transfusion needs," Sarah Gheuens, chief medical officer and head of R&D at Agios, said in a statement.

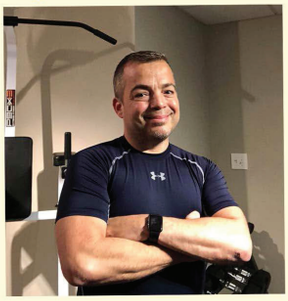
Gheuens said the company plans to submit a marketing application in the U.S. by the end of the year, which will include data from both trials. Pyrukynd was approved by the FDA in 2022 to treat hemolytic anemia in adults with pyruvate kinase (PK) deficiency.

Agios is also looking to submit marketing applications for the drug in Europe and the Gulf Cooperation Council countries, which include Saudi Arabia and the United Arab Emirates.

It's Never Too Late to Start Being Physically Active

John didn't become physically active until his mid-30s, when he began to gain weight, develop high blood pressure, and suffer from extreme tiredness. He decided to begin an exercise program to improve his health – and now he's in the best shape of his life!

Becoming fit was such a life-changing experience for John that he now coaches other people to help them achieve their health and fitness goals.



Any amount of physical activity provides health benefits, so do what you can based on your abilities after consulting with your doctor. You don't need to adopt a formal exercise program – you can easily get started simply by walking.

Find activities that get you moving and that you enjoy and make them a regular part of your life!

This message was developed as part of Cooperative Agreement #5NU27DD001150-04-00 from the Centers for Disease Control and Prevention.

Cooley's Anemia FOUNDATION
Leading the Fight Against Thalassemia
www.thalassemia.org

Imagine if your dollar is the one that helps us find a cure for all? Please help us in our fight against thalassemia by donating to ensure that patients get the care and support they need to live beyond 70. **Scan the QR code to donate today**

LIFELINE

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:

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Please return to:
Cooley's Anemia Foundation, 330 Seventh Avenue, Suite 200, New York, NY 10001

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

CELEBRATING 70 YEARS OF HOPE & CARE

The Cooley's Anemia Foundation is proud to celebrate 70 years of leading the fight against thalassemia. For over 70 years, CAF has been a steadfast source of support for thalassemia patients and their families.

In 1954, Fank Ficarra began organizing neighborhood blood drives when two of his young children were diagnosed with thalassemia major. Frank realized much more was needed to continue the fight so he gathered other parents of thalassemia patients in the back of a butcher shop to plant the seeds of what The Cooley's Anemia Foundation would eventually become.

Since that night CAF has grown into a national and international force with an extraordinary record of accomplishments. Between Fellowship programs for thalassemia research and many life changing resources for patients with thalassemia, CAF continues to be a stronger and supportive partner for families living with thalassemia.

What began as the story of one man's family is today the story of many families working together toward a common goal, finding a universal cure for all. CAF continues to fight for better treatments and resources for all people living with thalassemia. The goal is for all patients to live 70 years and beyond.

Through our 70 for 70 fundraising campaign, we've managed to raise over $500,000 thanks to our incredible supporters and sponsors. Your support is literally our lifeblood.

Thank You to Our Sponsors:

- The Peter Chieco Family (A Brighter Future Today)
- James & Gina Giordano Foundation (A Brighter Future Today)
- The Robert Ficarra Family (CAF Champion)
- Agios Pharmaceuticals (CAF Champion)
- Order of Sons & Daughters of Italy in American (CAF Champion)
- Carmine & Jenine Abruzzo (Ambassador of Hope)
- Chiesi USA (Ambassador of Hope)
- Ann Shuch (Patient Supporter)
- Laurie Pizzo (CAF Cheerleader)
- Columbus Citizens Foundation (Cheerleader)
- National Distribution Alliance (CAF Cheerleader)

Testing for Low Bone Mass Helps Prevent Fractures.

People with thalassemia have a greater risk of bone fracture due to low bone mass. Early identification of reduced bone mass may lead to strategies that can reduce the risk of fracture.

People with thalassemia should have a bone mass test (sometimes called a bone mineral density test) by age 10 and should repeat it annually or as needed. This applies to thalassemia patients whether they are transfused or non-transfused.

Early testing can help keep bones healthier and stronger. Talk with your doctor to learn more.

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Leading the Fight Against Thalassemia
www.thalassemia.org

PATIENT INCENTIVE AWARD WINNERS

The following were among recipients of 2024 CAF Patient Incentive Awards.

- Faye Barekat
- Marc Celestino
- Dara Chanthavisay
- Akash Chaurasia
- Eric Chiang
- Alexander Cui
- Meghan Frandsen
- Sevda Gerger
- Aeshah Nadeem
- Adrian Ng
- Nabil F Rahman
- Wanda Sihanath
- Edwin Tan
- Dan Vuong

The following were among recipients of 2024 CAF Incentive Awards for Children of Patients.

- Isabella Abruzzo
- Ryan Colasanti
- Amanda Cosentino
- Emma Marchese
- Kaylin Marchese
- Seema Anjali Sawh
- Victoria Schevon
- Isabella Shapiro
- Paul Vitaliti

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70 Years of Leading the Fight Against Thalassemia
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All contributions are tax-deductible.