



WINTER 2023

LIFELINE

COOLEY'S ANEMIA FOUNDATION • LEADING THE FIGHT AGAINST THALASSEMIA

RALPH COLASANTI ELECTED NATIONAL PRESIDENT OF CAF



History was made April 23, 2022 at the annual board meeting of the Cooley's Anemia Foundation (CAF) when Ralph Colasanti was elected the first person with thalassemia to serve as National President of the Cooley's Anemia Foundation.

"We are very pleased to have reached this historic moment and know that Ralph's talents, skills, knowledge, and expertise will serve him well in this position," says Peter Chieco, immediate Past National Pres-

ident of CAF. "Of equal importance, being a patient himself will provide a fresh perspective. No matter how deep the connection that I and our previous Presidents have with the thalassemia community, it is not quite the same experience as actually being a person who was born with and has lived with thalassemia for their entire lives."

"The fact that I am able to serve in this capacity speaks to the tremendous efforts of The Cooley's Anemia Foundation throughout the years," says Ralph Colasanti. "Those that served before me helped propel many advances in the field of thalassemia over the last 20-30 years. Their commitment, with support from our donors, brought forth dedicated clini-

"When I was diagnosed, the outlook was grim, a life expectancy into my late teens, early twenties, with many warning me that living a normal life - having a family, working, living very far into my adult years - was not likely, but faith brought me all that and more. And while there are still many mountains a person with thalassemia has to climb, we now are in a position where a well-managed patient can enjoy many of the same things as a person not living with thalassemia. It's a challenge, but I and others like me have learned to never give up. And definitely don't stop believing!"

– Ralph Colasanti

cians and researchers who are experts in thalassemia, not only advancing our quantity of life but the most important quality of life. These medical advances and the need for a safe blood supply are tirelessly promoted and supported by CAF.

Ralph is a financial coach and IT consultant at Holy Angels School. He and his wife are the parents of a son and a daughter and recently welcomed their first grandchild.

Partial funding for this issue of Lifeline is provided by Bristol Myers Squibb.

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

DISCLAIMER: The information in this publication is for educational purposes only and is not intended to substitute for informed medical advice. You should not use this information to diagnose or treat a health problem or disease without consulting a qualified health care provider. The Cooley's Anemia Foundation strongly encourages you to consult your health care provider with any questions or concerns you may have regarding your condition.

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

FROM CAF NATIONAL PRESIDENT RALPH COLASANTI



When I get a bag of blood, I don't know who it came from. I'm just thankful that there were enough people out there who are willing to roll up their sleeves and give a part of themselves to help a person like me.

But in fact, the unit of blood that I need can't come from just any person who donates. First, because it has to match my specific blood type. Second, when you get

transfused every few weeks like we people with thalassemia do, your blood develops antibodies that make it harder to find a unit of blood that will be a match. So the blood bank has to test and go through many, many units of donated blood to find one that will work for me – so in fact, it takes many, many people donating blood so that I can get the one bag that I need. (Actually, I need more than one bag of blood when I get transfused, but you get the idea.)

So it really does take a village, as they say. And that applies not just to blood donations and transfusions but to everything that CAF is involved in. We are a small foundation doing great

things, but we can only do those big things because of the thousands of people who support us. Everyone who attends a Gala or dinner dance or golf outing for CAF, buys a raffle ticket, makes a donation – you make it possible for us to do things like:

- offer our new program for thalassemia patients 50 and older who are striving to maintain their health
- provide assistance to thalassemia patients (and their children) who are pursuing higher education
- support patients traveling to a center for comprehensive care
- recognize the value of a family that adopts a child with thalassemia
- fund valuable medical research programs
- convene an impressive international medical symposium on thalassemia
- offer an amazing patient-family conference that is literally life-changing for so many who attend.

All this is more happens because of people like you who know of the challenges of living with thalassemia and take that extra step of helping CAF in its fight against thalassemia.

You are our lifeblood.

There's so much more we want to do – and we need your help, to do it! Any support is welcomed.

Sincerely,

Ralph Colasanti
National President

FDA APPROVES FIRST GENE THERAPY FOR PEOPLE WITH TRANSFUSION-DEPENDENT BETA-THALASSEMIA

On August 17, 2022, the FDA approved ZYNTEGLO, a one-time gene therapy custom-designed to treat the underlying genetic cause of beta thalassemia in adult and pediatric patients who require regular red blood cell (RBC) transfusions. Zynteglo is a product of bluebird bio.

The Cooley's Anemia Foundation applauds the FDA's historic approval of this process for people with beta thalassemia who require regular red blood cell transfusions. The availability of a one-time gene therapy which offers the possibility of transfusion independence opens up new and exciting opportunities for those who are medically eligible to receive this treatment option. While advances in treatment have been of enormous benefit to those with beta-thalassemia, a potentially curative therapy may offer a true life-changing experience.

This FDA approval offers people with beta-thalassemia the possibility of freedom from burdensome regular red blood cell transfusions and iron chelation and is a milestone in the treatment of thalassemia.

ZYNTEGLO works by adding functional copies of a modified form of the beta-globin gene into a patient's own hematopoietic (blood) stem cells (HSCs) to allow them to make normal to near normal levels of total hemoglobin without regular RBC transfusions. The functional beta-globin gene is added into a patient's cells outside of the body (ex-vivo), and then infused into the patient. Though ZYNTEGLO is designed to be administered to the patient once, the treatment process is comprised of several steps that may take place over the course of several months.

Due to the complex nature of gene therapy, ZYNTEGLO will be available exclusively at Qualified Treatment Centers (QTCs). Information on bluebird's QTC network, as well as personalized support focused on the needs of each patient throughout their treatment journey and information on insurance coverage and access will be available through bluebird's patient support program, my bluebird support. Patients can call 833-888-NEST (833-888-6378) for more information, and additional details are available at mybluebirdsupport.com.

CAF EARNS SPOT ON GREAT NONPROFITS TOP RATED 2023 LIST!

We're extremely excited to announce that The Cooley's Anemia Foundation was recognized by Great NonProfits in 2023! The Foundation's hard work and dedication for all thalassemia patients has resulted in Great NonProfits listing CAF as a Top-Rated non-profit organization in 2023.

Great NonProfits, 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 non-profit 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 also 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 2024!



CDC LAUNCHES NEW THALASSEMIA TOOLKIT FOR HEALTHCARE PROVIDERS

The Centers for Disease Control and Prevention (CDC) recently launched a new Thalassemia Healthcare Provider Resource Toolkit. This free toolkit is filled with the following free resources for providers caring for people living with thalassemia:

- **Continuing Medical Education Offerings and Webinars:** Find recordings of CDC and the Cooley's Anemia Foundation's Virtual Thalassemia Grand Rounds, CDC-funded webinars on thalassemia, and other continuing medical education offerings.
- **Scientific Articles and Features:** View the latest research findings and special features on thalassemia published by CDC authors or funded by CDC.
- **CDC Activities Related to Transfusion Complications in Thalassemia:** Learn about CDC's work on transfusion complications monitoring and how it is helping us learn more about the health problems that can occur after people with thalassemia receive blood transfusions.
- **Resources on Newborn Screening:** Find resources and learn about CDC's work in newborn screening for thalassemia.
- **Thalassemia Treatment Centers:** Find a map with information on select specialty thalassemia treatment centers in the United States.

- **Resources for Thalassemia Care, Treatment, and Management:** View resources on best practices for the management of people with thalassemia.
- **Clinical Thalassemia Management Checklists:** Find evidence-based checklists that were developed and reviewed by the scientific medical advisory board of the Cooley's Anemia Foundation.
- **Thalassemia Transfusion Resources:** View a collection of thalassemia transfusion resources for healthcare providers and people living with thalassemia and their families.
- **Other Clinical Management Resources:** Find links to other resources on clinical management issues in thalassemia.

WHAT YOU CAN DO:

- Visit <https://bit.ly/CDCThalToolKit> to access these resources and share this toolkit with your colleagues who might find this information helpful.
- Learn more about thalassemia by visiting <https://bit.ly/CDCThalassemia>
- Learn about CDC's work in monitoring complications after people with thalassemia receive blood transfusions.
- Stay current on the latest news and updates by following us on Twitter @CDC_NCBDDD.

CAF PROVIDES GRANT FUNDING TO FOUR MEDICAL RESEARCH FELLOWSHIPS

We're proud to announce two new Cooley's Anemia Foundation Medical Research Fellowships and two renewal Medical Research Fellowships for the 2023-2024 grant cycle. The total amount of funding for the four research Fellowships is \$200,000.

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 – Dr. James Bieker, Dr. Lionel Blanc, Dr. Alan Cohen, Dr. Yelena Ginzburg, Dr. Stefano Rivella, Dr. Sujit Sheth - 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.

FIRST-YEAR FELLOWS



Dr. Yanan Sun is currently a postdoctoral fellow in Dr. Shuaiying Cui's Laboratory in the Department of Medicine at Boston University School of Medicine and Boston Medical Center.

Dr. Sun's research focuses on the reactivation of fetal hemoglobin (HbF) in adult erythroid cells, where

she made quite significant breakthroughs.

Dr. Sun is playing a leading role in her ongoing β -thalassemia and SCD-related project sponsored by Cooley's Anemia Foundation research fellowship. This project aims to study a new compound to induce fetal hemoglobin production. The focus of this project is its effect in a related hemoglobinopathy, sickle cell disease. They will also study it in normal human stem cells as project 2.



Dr. Premila P. Samuel of the University of Illinois at Urbana-Champaign will focus on uncovering hidden states in hemoglobin mis-assembly.

This project will use computational chemistry techniques to capture and characterize three-dimensional configurations and chemical interactions of states along hemoglobin

mis-assembly pathways that lead to thalassemia manifestations, critical information which traditional lab-based techniques have not been able to fully access.

The overall project goal is to develop a virtual platform that will be used to model thalassemia pathways in red blood cells and subsequently screen for drugs that counter the toxic products of thalassemia in a rapid and cost-effective manner.

SECOND-YEAR FELLOWS



Francesca Vinchi, PhD of New York Blood Center, is studying the role of Non-Transferrin Bound Iron (NTBI) and iron toxicity in Bone Marrow Transplant (BMT) complications, focusing on beta-thalassemia.

In the first 6 months of grant support, Dr. Vinchi showed that conditioning elicited NTBI negatively impacts BMT

outcome in wild type mice. She also showed that conditioning aggravates NTBI formation in thalassemic mice and that iron restriction using hepcidin mimetics prevents this effect.

In the second year of funding she proposes to address the impact of conditioning elicited NTBI and pre-existing iron overload on BMT outcome and their impact on peri-transplant toxicities in thalassemia.



Beth Apsel Winger, MD, PhD of the University of California, San Francisco/Benioff Children's Hospital will use a new technique to improve dose selection of deferasirox for patients with thalassemia.

In a study of 50 patients, Dr. Winger will develop a dosing algorithm that takes into account multiple clinical

factors such as age, body size, and liver and kidney function as well as evidence of inadequate control of iron stores and complications of deferasirox. She and her colleagues have already successfully applied this methodology, known as population pharmacokinetics, to other therapies such as conditioning therapy for bone marrow transplant.

2024-25 FELLOWSHIPS & GRANTS APPLICATIONS

To apply for 2024-25 Fellowships & Grants visit:

<https://bit.ly/CAFGrants2024>

CAF'S 2023 GALA RECOGNIZES FOUR HONOREES



CAF held its first in-person Gala since 2019 on October 18, 2023 and used the occasion to recognize four honorees for their commitment to the thalassemia community. Those selected as honorees were **Ralph Colasanti, CAF National President; The Ficarra Family; The Giordano Family; and Vertex Pharmaceuticals.**

With the theme of “Milestones and Miracles,” this year’s event was held at a new venue, Lavan’s Midtown, which enabled CAF to utilize full-wall projections to create an immersive experience. Louis Piscione provided expert guidance as our event planner, creating a more casual yet elegant ambience which allowed for much more social interaction – as well as dancing to a sensational band throughout the evening. Laurie Messina Pizzo, Frank Guzzone, and Bob Zeltmann, along with CAF Communications and Marketing Manager Paris Booker, oversaw the development of projections and videos, as well as new visual elements intended to celebrate CAF’s 70th anniversary in 2024. The result was a vibrant, engaging evening which flowed smoothly between program interludes and times for chatting, eating, and dancing.

Ralph Colasanti was honored for his many years of service to CAF in general and in particular to the inspiration he gives as the first individual with thalassemia to serve as the Foundation’s National President. Bob Ficarra, whose parents founded CAF in 1954 and who himself is a founding member of the Thalassemia International Federation, accepted on behalf of the Ficarra Family. Jenine Giordano Abruzzo spoke of her parents, Jimmy and Gina, and her sister, Vivian, and their role as ardent supporters of CAF while accepting for the Giordano Family. And Dr. Kilpatrick Carroll, a Vice President at Vertex Pharmaceuticals, spoke of how a Vertex employee with close personal ties to thalassemia helped impress upon the company the needs of the thalassemia community.

Vertex is currently developing a gene editing procedure for beta thalassemia patients.

Frank Somma, a former CAF President and a member of the Board of Directors, served as master of ceremonies.

CAF thanks all of our honorees and sponsors, as well as all who attended this wonderful evening. The Foundation also thanks the tireless Gala Committee and especially Gala Co-Chairs Peter Chieco and Frank Fusaro. Thanks to the efforts of all concerned, more than \$540,000 was raised for the fight against thalassemia.

A VERY SPECIAL THANK YOU TO OUR GALA SPONSORS

DIAMOND SPONSORS:

- The Chieco Family
- The Giordano Family
- The Ficarra Family
- Vertex Pharmaceuticals

SILVER SPONSOR:

- Chiesi Global Rare Diseases

SUPPORTERS:

- Agios
- The Alliance Pharmacy
- Hardan Consultants Inc.
- NDA – National Distribution Alliance
- NYS Orders Sons and Daughters of Italy in America
- Laurie Messina Pizzo

FRIENDS:

- Frank Fusaro
- Ann Shuch
- Frank Somma

2023 PATIENT FAMILY CONFERENCE UNITES THALASSEMIA COMMUNITY FOR A SUCCESSFUL EVENT



The 2023 Patient Family Conference held July 7-9, 2023 in Los Angeles was a resounding success, bringing together the thalassemia community in a powerful display of unity, learning, and support. The event, attended by thalassemia patients, their families, doctors, board members, and staff, took place over three action-packed days at the Hilton Los Angeles Airport.

DAY 1 - FRIDAY: THALASSEMIA 101 AND ADULT PATIENTS MEETING

The conference commenced on Friday afternoon with an enlightening session titled “Thalassemia 101” led by Susan Carson RN, MSN, CPNP from Children’s Hospital Los Angeles. This session laid the foundation for the conference, providing essential information to all attendees.

Later that day, an opening dinner was held, providing an opportunity for attendees to network and connect. The evening continued with a meeting specifically designed for adult thalassemia patients, fostering a supportive and inclusive atmosphere.

DAY 2 - SATURDAY: A DAY OF LEARNING AND INSIGHTS

Saturday started with a hearty breakfast and an opening statement by the CAF National President Ralph Colasanti. After inspirational words of encouragement to the thalassemia community, Ralph introduced Dr. Thomas Coates from Children’s Hospital Los Angeles. Dr. Coates highlighted the importance of iron chelation therapy in managing thalassemia.

Next John Wood, MD, PhD from Boston Children’s Hospital shared valuable insights on “PHT & Atrial Fibrillation in Thalassemia,” followed by Jennifer Eile, PNPO-BC from Boston Children’s Hospital and her informative session on essential blood transfusion tips.

We were honored to have Dr. Elliott Vinchinsky from UCSF Benioff Children’s Hospital in Oakland to give insights and information on alpha thalassemia major, followed by Dr. Tariq Ahmad on keeping your bones healthy as a thalassemia patient. Dr. Ashutosh Lal shared the new therapies and what’s here to come for thalassemia patients in the future.

During the midday break, the Care Walk committee featuring CAF National Treasurer Janice Cenzoprano, Radhika Sawh, Maria Saradpon, Yasmeen Anis and Jessica Horstmann provided an update on their endeavors, emphasizing the community’s collective efforts to organize and support the creative and engaging fundraiser.

After lunch, we had an amazing patient-provider dialogue with Dr. Tom Coates and Laurice Levine featuring insightful discussions. Following this, a passionate thalassemia patient panel led by CAF Patient Social Worker Kathleen Durst, brought real-life perspectives to the forefront of those attending. Each patient shared the trials and tribulations in dealing with thalassemia in their personal lives.

The afternoon continued with informative updates on Gene Therapy with e beta thalassemia patient Olivia Stahl and Dr. Mark Walters from UCSF Benioff Children’s Hospital in Oakland. Olivia shared her gene therapy treatment experience, shedding light on cutting-edge advancements.

To add a touch of fun to the event, a lively Thalassemia Quiz Show was held, hosted by Daniella Macolino, bringing participants together in a light-hearted yet educational environment on all things thalassemia related!

The first night concluded with a memorable Carnival Themed Dinner Dance, where patients and their families had an unforgettable time bonding and celebrating.



DAY 3 - SUNDAY: CLOSING ON A HIGH NOTE

Sunday morning commenced with a rejuvenating group Yoga session, setting the tone for the day. Following breakfast, the Children's Session Presentation took center stage. This was followed by our national CAF president Ralph Colasanti & Michelle Lahat from Children's Hospital Los Angeles leading discussions on Financial Planning and Insurance Issues in Thalassemia, providing crucial guidance for patients and families.

The event's finale consisted of separate thalassemia group sessions, allowing patients and families to interact, share experiences, and forge lasting connections.

As always, there were special sessions for children and for teen-agers. The teen participants this year engaged in activities including an improvisation class, a yoga session, and a "sip and paint" hour. They ended Saturday by creating an episode of the Thal Pals podcast which Agios Pharmaceuticals produces. (Special thank you to Agios for making this possible.)

The success of the 2023 Patient Family Conference promises an even brighter future for the thalassemia community. We are very excited for the conference in 2024, which will take place in Atlanta! Stay tuned for the official announcement.

THANK YOU TO OUR CONFERENCE SPONSORS

Gold Sponsors

- bluebird bio
- Chiesi Global Rare Diseases
- Vertex

Silver Sponsor

- Bristol Myers Squibb

Bronze Sponsors

- Agios
- Editas Medicine

CAF RECEIVES INVALUABLE SUPPORT FROM SAVOY FOUNDATION

The Cooley's Anemia Foundation is honored to have received support from the prestigious American Foundation for Savoy Orders at its 2022 Ballo di Savoia on December 17, 2022 at the University Club in New York City. The Foundation donated a very generous \$50,000 to CAF to support its work on behalf of children and youth with thalassemia.

The American Foundation of Savoy Orders is the philanthropic arm of the American Delegation of Savoy Orders in America. The primary mission of the American Delegation of Savoy Orders in North America is to perpetuate the principles, traditions and humanitarian work of the Dynastic Orders of the Royal House of Savoy. As described by Timothy Michael Cardinal Dolan, Archbishop of New York, the American Foundation for Savoy Orders "has a proud tradition of charity and generosity to our human family through educational, social, medical and humanitarian assistance."

CAF National President Ralph Colasanti was invited to speak at the Ballo di Savoia to thank the Foundation for including CAF as a beneficiary of the evening's donations. A Chance in Life, another worthy non-profit which focuses on empowering at-risk children and youth, was also a beneficiary of the Savoy Foundation's largesse.

A member of the CAF National Board and President of the American Foundation of Savoy Orders, Joseph Sciamè, recently commented, "The members and friends of the American Foundation of Savoy Orders have been pleased to support the work of the Cooley's Anemia Foundation and especially knowing that this support is having an impact on children with this challenging blood disorder. We all look to a future resolution for the children who are our future and we support the CAF under our mission: Chivalric Causes for Children."

CAF is fortunate to again be a beneficiary of the upcoming 2023 Ballo di Savoia, which will be held on Saturday, December 9, 2023. Information about the event, including the opportunity to order tickets, is available at:

<https://bit.ly/2023SavoyBall>

PATIENT PROFILE: RADHIKA SAWH

“I have worked hard my entire life to not let thalassemia define me or prevent me from living my life to its fullest.”

– Radhika Sawh

These are words spoken by Radhika Sawh, a woman born with beta thalassemia major. Radhika’s spirit and determination have enabled her to face the many challenges presented by thalassemia—starting with the need for regular blood transfusion.

Radhika began what would become a lifetime of blood transfusions when she was 18 months old. “At first, I received less than 1 unit (approximately 1 pint) of blood cells, because I was so small,” she says. “But by the time I was 3 years old, I needed 2 full units every 2 months. And as I became older and continued to grow, my blood requirements also increased. By age 4, I needed blood every 6 weeks, and by age 6, every month. During high school, I required blood transfusions every 3 weeks, and by the time I was in my mid-20s, I needed blood every other week.” Radhika once figured out that by the time she was 45 years old, she had received more than 1,500 units of blood!

Radhika was fortunate to be diagnosed with thalassemia right after she was born, so that her doctors could monitor her and know when to begin transfusions. One reason that she was diagnosed properly was because her older brother was born with thalassemia as well. Unfortunately, Radhika never knew her brother. He passed away at age 7, just 3 months before Radhika was born.

But her brother’s experience with thalassemia also influenced Radhika’s life beyond her early diagnosis. Radhika is a board-certified genetic counselor who gathers personal and family health information from people and helps them understand their personal risk of having genetic conditions (such as thalassemia). In this role, Radhika empowers others with information to help them make informed decisions about their health.

“I was inspired to pursue this career based on my parents’ experience with my older brother’s diagnosis with thalassemia major,” Radhika explains. “They were under the impression that because they already had one child with thalassemia major, their next child would not be at risk. They were devastated when soon after I was born it became clear that I also had thalassemia major, the disease which had only months before taken the life of their firstborn. A visit with a genetic counselor would have ensured my parents understood that because they were both carriers, they had a one in four chance with each pregnancy of having a child with thalassemia major.”

Despite her need for regular transfusions, Radhika was able to go to college, continue her education in graduate school, and

enjoy a fulfilling career. She also is married and has a daughter who is herself now in college. These options are now more common for people with thalassemia than they once were.

“When I was growing up, I didn’t know anyone with thalassemia older than 30 years of age, let alone anyone who was married and had children,” Radhika says. “Today, that’s all changed.”

Radhika is thrilled to see so many changes in thalassemia management and treatment occurring during her lifetime and to realize how different things are now. When asked what advice she has for others with thalassemia, Radhika responded, “Having thalassemia is not a death sentence. Most people with this blood disorder are able to lead full lives, pursue careers, and have families of their own. It all depends on how you manage your disorder. You will have to learn to plan your life around your transfusions, stay on top of your chelation and advocate for yourself, but as long as you do, you can live the life you want, without limitations.”

Despite all she has accomplished, Radhika acknowledges that one thing hasn’t changed: the need for regular blood transfusions.



“I wish more people who are eligible to do so would donate blood. It is very scary for people like me who depend on blood transfusions to hear about blood shortages, because this can mean life or death for us. So, if you are able, please consider donating blood!”

– Radhika Sawh

CDC and CAF thank Radhika for sharing her personal story.

This article originally appeared online as part of the CDC’s “Real Stories for People Living with Thalassemia.”

CARE WALK 2023: YOUR WALK YOUR WAY



Care Walk 2023 was a great success!! This year, with the help of social media, we were able to reach out to new team captains and donors that helped us raise so many funds for thalassemia research!

Participants had the freedom to choose when and where they wanted to walk, making it a unique and meaningful experience for everyone. This year we had a record of 34 teams and over 300 attendees that signed up for the purpose of advocating for the lives of many battling thalassemia. We had walks in NYC's Labor Day Parade, Los Angeles, Fort Lauderdale, Arizona, Long Island, Chicago, New Jersey, Delaware and much more!!

HERE ARE THE TOP 5 TEAMS THAT RAISED THE MOST FUNDS THIS YEAR:

- Connie's Crew organized by CAF Treasurer Janice Cenzoprano
- Team Ficarra organized by The Ficarra Family
- Chicago CAF organized by Arianna Bonomi & Teresa Tomaino
- CHOP PENN Warriors organized by CAF National President Ralph Colasanti
- Grateful Steps organized by new team captain Gina Ortega

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.

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 would love to support or create your own Care Walk in 2024, 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.

It's all about making 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.

Special thanks to all the hardworking team captains, sponsors, supporters and donors that helped make Care Walk 2023 a success! Together, we can make a lasting impact and bring a brighter future to those living with thalassemia.

50+ HEALTH MAINTENANCE AWARD

The Cooley's Anemia Foundation (CAF) recognizes 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 to Thalassemia patients (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

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
- To 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, 2023 and June 30, 2024

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, 2023 and June 30, 2024 for expenses occurred during that period (between July 1, 2023 and June 30, 2024).

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.

CDC ONLINE PROGRAMS FOCUS ON THALASSEMIA

CAF is fortunate to be engaged in a collaborative agreement with the Centers for Disease Control and Prevention (CDC)'s Division of Blood Disorders, the aim of which is to provide health promotion and prevention programs for the thalassemia community. As part of that agreement, CAF works closely with CDC on webinars and virtual grand rounds which help to educate health care providers about evidence-based practices related to thalassemia care and management.

The CDC Public Health Webinar Series on Blood Disorders focuses on issues of importance to the blood disorder community. CAF is fortunate to have been able to offer assistance in preparing several sessions that focus on thalassemia. The most recent session was held June 22, 2023 on the topic "Emerging Trends in Gene Therapy: Thalassemia as a Case Study." Stefano Rivella, PhD, of Children's Hospital of Philadelphia, and Sujit Sheth, MD, of Weill Cornell Medicine, were presenters; Muin J. Khoury, MD, PhD, Director, Office of Genomics and Precision Public Health at CDC, moderated. There have been many advances in the gene therapy field in recent years; the first gene therapy process for thalassemia was approved by the FDA in 2022, and there are several clinical trials in gene therapy for thalassemia that hold promise. The information presented by Dr. Rivella and Dr. Sheth shed a great deal of light on both the current state of gene therapy and thalassemia and what lies ahead.

CAF also helps prepare a series of Virtual Thalassemia Grand Rounds, a virtual forum for healthcare providers to learn the latest in the diagnosis, treatment, and management of thalassemia, as part of its collaborative agreement with the CDC. The latest entry was on the topic of "Beyond Iron: Understanding Cardiac Complications in Thalassemia." Held on September 21, 2023, it featured John Wood, MD, PhD, of the Keck School of Medicine of USC, as presenter and Gregory Kurio, MD, of University of California at San Francisco, as moderator. Dr. Wood focused specifically on pulmonary hypertension and atrial fibrillation and how they may occur in individuals with thalassemia.

CAF is grateful to CDC for the opportunity to collaborate on these exciting programs which help to inform health care providers about recent advances in management of thalassemia. We especially thank our Project Officer, Cynthia A. Sayers, for her guidance on all aspects of our collaborative agreement.

SUFFOLK CHAPTER DINNER DANCE RAISES OVER \$500,000



The Suffolk Chapter of CAF held its 53rd Annual Gift of Life Ball at Villa Lombardi's Catering Hall in Holbrook, Long Island on Thursday, June 29, 2023. This year's honoree was Michael Lochren of Jenco Ready Mix.

Michael and his Dinner Dance Committee raised \$520,000 to assist the Foundation in patient services and research. Over 300 people attended the event which featured the ever-popular singer, Christopher Macchio. Featured speaker was President of the Cooley's Anemia Foundation, Ralph Colasanti.

The Suffolk Chapter's next Annual Ball will be held on June 13, 2024.

Being adherent to thalassemia treatment has a big payoff – a longer life.

Studies have shown that people with thalassemia who follow the recommended treatment plans for thalassemia have better health outcomes than people who don't.



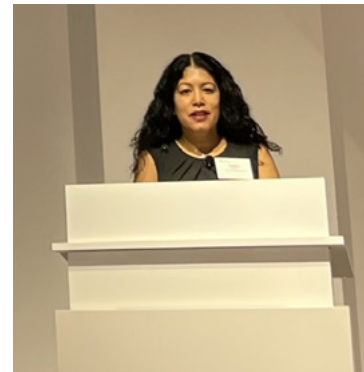
So even if you can't tell that your treatment is making a difference, your body can!

Sticking with transfusions, chelation and other treatments can be difficult – but it's worth it.

This message was developed as part of Cooperative Agreement #1U27DD001150-01 from the Centers for Disease Control and Prevention.

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

11TH COOLEY'S ANEMIA SYMPOSIUM EXPLORES STATE OF THALASSEMIA TODAY



More than 250 clinicians, scientists, and other members of the thalassemia community gathered for the 11th Cooley's Anemia Symposium, convened by the Cooley's Anemia Foundation (CAF) in collaboration with the New York Academy of Sciences (NYAS) from October 17-20, 2022 in New York City.

The latest in this landmark series of international meetings, which began in 1964, featured 14 sessions covering 62 presentations on a wide range of topics of importance to those working in thalassemia. The agenda was devised by a Scientific Organizing Committee consisting of Melanie Brickman Borchard, PhD, MSc, of NYAS; Craig Butler, National Executive Director, CAF; Janet Kwiatkowski, MD, MSCE, Children's Hospital of Philadelphia; Yelena Z. Ginzburg, MD, Mount Sinai; Stefano Rivella, PhD, Children's Hospital of Philadelphia; Alexis A. Thompson, MD, MPH, Children's Hospital of Philadelphia; and Vip Viprakasit, MD, DPhil, Siriraj Hospital, Mahidol University.

The agenda was far ranging and covered topics including Clinical Management and Therapies; Clinical Complications; Novel Therapies; Gene Therapy; Regulation of Iron; Alpha Thalassemia; Pregnancy and Fertility; Transplantation; Patient Reported Outcomes; and Novel Mechanisms of Globin and Erythroid Iron Regulation. The Day 1 Keynote Address was delivered by Alan R. Cohen, MD of Children's Hospital of Philadelphia and focused on "The Big Picture – Thalassemia: The Changing Clinical Picture." A Day 2 Keynote Address concerned "History of Gene Therapy for Thalassemia" and was delivered by Giuliana Ferrari, PhD, of Scientific Institute San Raffaele.

To close the Symposium, Michael Angastiniotis, MD, DCH of Thalassemia International Federation spoke on "Looking to

the Future: Addressing Unmet Needs and Challenges."

"The information presented at the 11th Cooley's Anemia Symposium highlights the tremendous advances that have occurred in thalassemia, not only since the first of these meetings in the 1960s but even since the 10th Symposium in 2015," says Janet Kwiatkowski, who acted as Chair of the Scientific Organizing Committee. "The breadth of scientific research and its clinical application is impressive and very encouraging, and will translate into improved patient outcomes. I am very grateful to all of the speakers who shared their expertise and knowledge at this Symposium, as well as to all those who attended and added their own views and experiences to the discussion. I'd like to especially acknowledge the members of the Planning Committee who worked diligently to organize the agenda and select an excellent array of speakers, and Melanie Brickman Borchard and everyone at NYAS who made sure the entire event ran smoothly."

Vertex Pharmaceuticals and Chiesi Global Rare Diseases supported the Symposium as Diamond Sponsors. Agios provided support as a Platinum Sponsor and bluebird bio and Bristol Myers Squibb as Gold Sponsors. Silver sponsorships were provided by Editas and Hemanext. CAF also thanks the National Heart, Lung and Blood Institute (NHLBI) for support under Award Number R13HL165804.

The Foundation extends its heartfelt thanks to all of the members of Scientific Organizing Committee; the many speakers who shared their expertise; the staff of NYAS; our sponsors; and all who attended the event and shared their thoughts and experiences in any way.

PATIENT INCENTIVE AWARD WINNERS

The following were among recipients of 2023 CAF Patient Incentive Awards.

- Yasmeen Anis
- Marc Celestino
- Akash Chaurasia
- Christina Chhim
- Sevda Gerger
- Anjelica Lapi
- Tobias Larkin
- Natalie Maino
- Grace Mei-Peng Richard
- Hannah Richards
- Kallie Shanahan
- Edwin Tan
- Katrina Wagar

The following were among recipients of 2023 CAF Incentive Awards for Children of Patients.

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

For information about the 2024 awards visit:

<https://bit.ly/24CAFIncentive>

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!

Pregnancy in Thalassemia

Through better management of iron overload, more and more women with thalassemia are able to become pregnant and give birth to healthy babies. If you are pregnant and have thalassemia, your pregnancy should be managed by a multidisciplinary healthcare team. Women with heart disease or significant cardiac iron are at increased risk during pregnancy because of added stress on the heart and circulatory system.



Maintaining an appropriate transfusion schedule and iron management before pregnancy is important. Blood transfusion requirements typically increase and iron chelation therapy is usually discontinued during pregnancy. Work closely with your hematologist to determine the best chelation plan for you if you are pregnant or planning on becoming pregnant.

The healthier you are before you become pregnant, the better things will be for both you and your baby!



See the 2012 Standards of Care Guidelines for Thalassemia published by Children's Hospital and Research Center Oakland, Section 18.1, for further recommendations on Pregnancy in Thalassemia.

In Memoriam

We regretfully report the loss of patients

AMY SERLIS
ELEANOR BALDWIN-BROWN
MICHAEL PASCETTA
ADAM SURANI
JOSEPH LOCICERO
MICHAEL FRANK

and extend our sympathies
 their friends and families.

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:

- | | | |
|---|---|---|
| <input type="checkbox"/> Last Will and Testament | <input type="checkbox"/> Gift of Real Estate | <input type="checkbox"/> Transfer on Death (TOD) on Account |
| <input type="checkbox"/> A Codicil in a Will | <input type="checkbox"/> Retirement Plan
Beneficiary Designation | <input type="checkbox"/> Contribution to
Endowment |
| <input type="checkbox"/> Individual Retirement
Account Charitable Rollover | <input type="checkbox"/> Charitable
Remainder Trust | <input type="checkbox"/> Other _____ |
| <input type="checkbox"/> Charitable Gift Annuity | <input type="checkbox"/> Charitable Lead Trust | |
| <input type="checkbox"/> Gift of Life Insurance | | |

We/I have made our/my designation to

Cooley's Anemia Foundation, Tax ID # 11-1971539, 330 Seventh Avenue, Suite 200, 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.

NAME(S) _____

ADDRESS _____

CITY _____ STATE _____ ZIP _____

PHONE _____ E-MAIL _____

SIGNATURE _____ DATE _____

SIGNATURE _____ DATE _____

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

CAF NATIONAL PRESIDENT TO BE HONORED AT WINTER CHARITY BALL

Ralph Colasanti, National President of CAF, has been selected as one of the honorees for the upcoming 2024 Winter Charity Ball, held by the New York State Grand Lodge Foundation, Inc., Order Sons and Daughters of Italy in America. The event will take place on January 26th at Leonard's Palazzo of Great Neck.

annual event enables the Foundation to continue its philanthropic mission in supporting charitable causes as in past years.

The Order Sons and Daughters of Italy in America has been an avid supporter of CAF for decades. At the recent national OSDIA meeting in August, the brothers and sisters donated more than

“Ralph’s vigor and leadership of the Cooley’s Anemia Foundation, along with his winning battle against thalassemia, make him truly a man to admire and honor”

– Luigi Squillante, NYS 2nd Vice President and State Fundraising Chairperson.

“I am overwhelmed that the New York Grand Lodge has selected me as one of this year’s honorees,” says Ralph Colasanti. “CAF has no greater friend than the brothers and sisters of this amazing organization.”

The objective of the NYSOSDIA Winter Charity Ball is to help provide funds for various charitable endeavors. Support of this

\$120,000 to the fight against thalassemia. The New York State Grand Lodge and its member lodges are among the most dedicated and generous supporters of our patients and families.

For further information about the 2024 Winter Charity Ball, please contact Winter Charity Ball Chairperson Luigi Squillante at the office of the New York Grand Lodge: nysosia@aol.com.

CAF BOARD OF DIRECTORS

Ralph Colasanti
President

Joseph Vigliotti, Esq.
Vice President

Janet Kwiatkowski, MD, MSCE
Medical Advisory Board Chair

Maria Hadjidemetriou
Secretary

Janice Cenzoprano
Treasurer

EXECUTIVE DIRECTOR

Craig Butler

LIFELINE EDITOR

Paris Booker
Marketing & Communications Manager

CAF BOARD MEMBERS

Carmine Abruzzo

Tracy Antonelli

Josephine Bila, MSW

Cammie Brandofino

Shirley Cammilleri

Amy Celento

Julia Cenzoprano

Mary Ann Cervoni-Iaia

Thomas Cheng

Peter Chieco

Alan Cohen, MD

Sarah Baqueri Connolly

Terri DiFilippo

Joseph DiTrapani

Anthony Ferrino

Robert Ficarra

Antoni Foe

Frank Fusaro

Anish Goel, PhD

Dean Hernan

Christine Horton

Michelle Chieco Lenz

Ben Li

Laurie Messina

Cindy Morrison

William Pisano

Paul Polo

Michael Pouchie

Michael Presto

Thomas A. Rotolo

Phil Rutigliano

Pranav Saha

Radhika Sawh

Joseph Sciamè

Frank Somma

Frank Tidona

Teresa Tomaino

James Ventola

Anthony J. Viola, CPA

Tom Watral

Joseph Zuraw

Nunzio Cazzetta, Emeritus Member

Hilary Cooley, Honorary Member

RETURN SERVICE REQUESTED

Non-Profit Org.
U.S. Postage
Paid
Hackensack, NJ
Permit No. 1534

WITH YOUR HELP, THE CURE IS WITHIN REACH!



BE A CHAMPION OF HOPE. YOUR DONATION
MAKES A DIFFERENCE!

I WANT TO MAKE A DIFFERENCE BY MAKING A TAX-DEDUCTIBLE CONTRIBUTION OF:

\$35 \$55 \$100 \$250 \$500 OTHER \$ _____

NAME _____

ADDRESS _____

CITY/STATE/ZIP _____

E-MAIL _____

Please make all checks payable to the **Cooley's Anemia Foundation**.

MASTERCARD VISA AMEX

CARD # _____ EXP. _____

Mail to: Cooley's Anemia Foundation
330 Seventh Avenue, #200 New York, NY 10001

All contributions are tax-deductible.

