



SUMMER 2021

# LIFELINE

COOLEY'S ANEMIA FOUNDATION • LEADING THE FIGHT AGAINST THALASSEMIA

## CARE WALK GOES VIRTUAL

Just as we did last year, CAF is offering a virtual Care Walk for 2021! Despite the challenges of the pandemic, thousands of caring supporters across the U.S. joined us last year for our first-ever Virtual Care Walk in honor of all those living with the challenges of thalassemia, raising over \$221,000 for the Foundation. Care Walk is one of the Foundation's most important activities, helping the members of our far-flung community feel unified and appreciative that so many people across the country are willing to support them.

In response to the pandemic, many of our Care Walk teams opted to walk privately with their families rather than meeting in large public gatherings to walk together. So to help us all feel a little more connected, we decided to create a virtual event last September. During the virtual event, supporters heard stories from patients young and old from different parts of the country and learned more about the Foundation's work on behalf of individuals with thalassemia. We received so much positive feedback that we are doing another Virtual Care Walk this year!

In addition to energizing our patients and families, Care Walk plays a huge role in enabling CAF to maintain and expand its important programs. Because of your support,

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## CAF'S FIRST VIRTUAL GALA A NOTABLE SUCCESS



The Cooley's Anemia Foundation's 2020 Gala took place on October 28, 2020 and was held entirely online to ensure the safety of all attendees during the pandemic. In addition to our usual Gala program, the evening included a live auction and a variety of activities, videos and entertainment that made the gala a memorable and exciting online experience. By the end of the evening, our supporters raised almost \$600,000 to ensure a brighter, healthier and fuller future for all thalassemia patients.

Anthony Scaramucci, Founder and Co-Managing Partner of SkyBridge Capital, accepted the CAF Humanitarian of the Year Award. He is a member of the Council on Foreign Relations, vice chair of the Kennedy Center Corporate Fund board, a board member of both The Brain Tumor Foundation and Business Executives for National Security, and a Trustee of the United States Olympic & Paralympic Foundation. A native of Long Island, Scaramucci holds a Bachelor of Arts degree in Economics from Tufts University and a Juris Doctor from Harvard Law School.

"We are so blessed to have had Anthony's support and generosity during an extremely difficult time for fundraising," said Peter

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Partial funding for this issue of Lifeline has been provided through a grant from Novartis Pharmaceuticals.

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## FROM CAF NATIONAL VOLUNTEER PRESIDENT PETER CHIECO

As I sit at my desk working from home, it is easy to see how the world has changed this past year and a half. Our Foundation has a long history going back to 1954 and has experienced many difficult periods in the past 66 years. But, none of us have ever been in a Pandemic before.

Despite this fact, your Foundation, continues to operate efficiently and for the benefit of patients. We have relied upon our strong governance structure, prudent cash reserves, outstanding staff, and innovative fundraising activities to continue to weather these trying times.

We know it has been a difficult year for our patients who rely on a safe supply of fresh donated red blood cells, while also worrying about catching the virus. I applaud their courage to venture out to the clinics, hospitals, infusion centers, doctor offices, ect., in managing their care.

Our staff has been up to the challenge in supporting their efforts, and is led by a very talented and hardworking executive director, Craig Butler. Craig and his team have met the unique challenges of our times head on and they are to all be congratulated for a job well done!

They have assisted a very dedicated executive committee, who continues to provide solid advice and support in helping governing the organization. In particular, I want to highlight our fundraising committees for both the Care Walk and Gala this



year. The Carewalk led by Janice Cenzoprano, and the Gala led by Frank Fusaro, both found a way to be successful in a virtual environment. They and their committee members did an amazing job in keeping the needed funds flowing in.

We especially want to highlight our Gala honoree this year, Anthony Scaramucci. Anthony and his wife Deidre and family, generously

supported our first ever virtual fundraiser. As our honoree, he showed tenacity and incredible strength of character in honoring his commitment made during a pre Covid world, to help our patients at the most critical time in our Foundation’s history. With their help, we had our most successful Gala ever!

Thirty-two years ago, my family and I became involved with the Foundation. It offered us hope for our daughter, support to follow best medical practices, a way to move forward positively, and it continues to do so. The relationship we share with the Foundation is a true blessing in our lives and it is available to everyone battling this disorder.

I ask that everyone touched by thalassemia, not only utilize the resources of the Foundation to the fullest, but, also, that you find creative ways to help us continue to grow. Together we have accomplished much already, but, our mission is still far from over. You have our promise to not rest until, every patient anywhere, can live their fullest life possible.

Thank you for your continued support.  
Peter

## UPDATED REQUIREMENTS FOR CAF’S TREATMENT TRAVEL SUPPORT PROGRAM

CAF is pleased to announce that it has revised the requirements for travel reimbursement 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.) There is no longer a minimum distance requirement for eligibility. Details and application can be downloaded at the following link: [bit.ly/ttsp-2020](https://bit.ly/ttsp-2020)

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## CAF AWARDS \$350,000 IN MEDICAL FELLOWSHIPS

The Cooley’s Anemia Foundation has awarded seven fellowships totaling \$350,000 as part of its 2021-2022 Medical Research Funding Program. Recommended by the Scientific Review Committee and approved by the CAF Board of Directors, the Fellowships represent funding for four new applicants and three second-year renewals. Each Fellowship is funded at the level of \$50,000 per year, renewable for a second year upon satisfactory progress achieved during the first year of funding.

### NEW FELLOWSHIPS

The four new applicants who received funding are:



**Phillip Doerfler, PhD**, St. Jude Children’s Research Hospital, Defining the mechanism of gamma-to-beta-globin gene switching and the genotoxicities associated with its manipulation by therapeutic genome editing. Inducing expression of fetal hemoglobin is a promising path to addressing thalassemia.

This study seeks to add to our understanding of the regulation of gamma to beta-globin gene switching and the safest, most effective genome editing approach(es) to inhibit this process.



**Christophe Lechauve, PhD**, St. Jude Children’s Research Hospital, mTORC1 inhibition for the treatment of  $\beta$ -thalassemia. Dr. Lechauve is focusing on a novel approach to alleviate  $\beta$ -thalassemia that focuses on inhibition of a protein called mTORC1, which he suggests will relieve the anemia by aiding the clearance of excess alpha globin chains.



**Mahesh Ramamoorthy, PhD**, Cleveland State University, Investigating the role of ATRX mediated subtelomere cohesion on alpha globin expression in ATRX syndrome. Alpha-thalassemia x-linked intellectual disability (ATRX) syndrome is a genetic condition in which alpha-thalassemia is associated with intellectual disability, muscle

weakness, and possibly other symptoms. The project proposes to investigate how mutations in the ATRX gene impact the production of  $\alpha$ -globin chains. The significance is the potential to develop approaches that could be used to either enhance  $\alpha$ -globin expression in  $\alpha$ -thalassemia or decrease  $\alpha$ -globin expression in  $\beta$ -thalassemia.



**Beth Apsel Winger, MD, PhD**, University of California, San Francisco/ Benioff Children’s Hospital, Establishing pharmacokinetically-driven, individualized dosing of deferasirox in transfusion dependent thalassemia. This proposal addresses the problem of treatment failure and drug-related adverse events with the iron chelator deferasirox. The

applicant will use a new technique to improve dose selection of deferasirox for patients with thalassemia.

### RENEWAL FELLOWSHIPS

The three Fellowships renewed for a second year are:



**Richard Coffey, PhD**, University of California, Los Angeles, Pathological Implications of Erythroferrone Excess In Thalassemia: Evidence From Novel Transgenic Mice. The main aim of the project is to uncover the role of high erythroferrone (ERFE), a hormone, in  $\beta$ -thalassemia. The project is expected to significantly extend our current knowledge about

the role of ERFE in iron homeostasis as well as in additional pathophysiologic aspects in the context of  $\beta$ -thalassemia.



**Merlin Nithya Gnanapragasam, PhD**, Cleveland State University, Investigating the post-transcriptional regulation of human fetal hemoglobin by the RNA binding protein PUM1. Increases in fetal hemoglobin can improve the clinical symptoms of  $\beta$ -thalassemia; the proposed studies will determine if modulating the levels of a protein called PUM1

might be a potential therapeutic strategy for fetal hemoglobin induction.



**Xia Xiao, PhD**, Massachusetts General Hospital, The hepcidin regulatory roles of hepatocyte transferrin receptor 1 in  $\beta$ -thalassemia.

The main aim of Dr. Xiao’s project is to uncover the contribution of a particular protein (TfR1) to the regulation of another protein, hepcidin. Because hepcidin plays a key role in how the body regulates iron, understanding what factors may affect it may lead to new ways of controlling hepcidin and thereby controlling iron loading in thalassemia.

CAF is excited to support these talented investigators and looks forward to the results of their studies.



## CAF SOCIAL WORKER KATHLEEN DURST HOSTS VIRTUAL GROUP SESSIONS

Members of the thalassemia community have faced many unique challenges since the onset of the COVID-19 pandemic. While CAF Social Worker Kathleen Durst has been leading occasional group sessions for patients and parents over the past 12 years, the need for support has greatly increased over the past year. In response to this increased need, Kathleen has been hosting weekly virtual mini group meetings since April of last year.

These group sessions are designed for individuals with thalassemia as well as parents of individuals with thalassemia. Some of these sessions are ongoing and are separated by age group, while others are one-time sessions focused on specific topics. Meetings take place virtually on Zoom and cover topics ranging from coping with stress associated with COVID-19 and best hygiene practices, to emotional well-being and how to manage children's behaviors during home school. One of the more popular sessions is a ten-minute breathing and mindfulness group session which is open to all ages and takes place every Thursday at noon, Eastern time. The overall feedback regarding these sessions has been very positive, and Kathleen will continue these sessions for the foreseeable future.

Ongoing group sessions are separated into age groups for patients who are 50-plus, 40-plus, and 20-plus. The purpose of these groups is to provide patients with the opportunity to connect with other individuals with thalassemia in a similar phase of life, to gain helpful tools on different topics, and to simply take a break from the day for self-care. Some sessions are hosted by Kathleen, and others are facilitated by members of the groups. For instance, the 20-plus session is co-facilitated with Priyanka Kumar and Daniella Macolino, two individuals with thalassemia in this age range. We encourage patients to participate in these groups if they haven't already joined.

Kathleen has also hosted several specialized sessions designed to address specific challenges unique to specific subsets of the community. For example, Kathleen partnered with Child Life Specialist Kia Ferrer to design and cohost a special session called "Transforming Worry" this past December. Worry is one of the many emotions that both children with thalassemia and their parents regularly confront, both in the hospital setting and in everyday life. Particularly during this time when many children are not attending school in person, emotions like stress and worry can intensify when combined with the isolation of these new circumstances. The focus of this group session was educating parents about how to recognize and transform worry in themselves, as well as how to coach their children in learning these skills.

Besides group sessions, Kathleen is also offering drop-in Tuesdays when patients or parents can spontaneously call, text or email her to get support about something happening in their lives or in the thalassemia community. She is available to provide general support, a 10-minute breathing exercise



or mindfulness exercise, and will listen to whatever is on your mind. She will continue these drop-in sessions throughout the year on Tuesdays from 12-5pm ET. If Tuesdays don't work for you, Kathleen is also available for pre-scheduled appointments. The drop-in sessions are just more casual and do not require scheduling in advance.

If you are interested in participating in a Tuesday drop-in session, a scheduled one-on-one session, or a group session, please **text Kathleen at 412-598-4283 or email [kdurst@thalassemia.org](mailto:kdurst@thalassemia.org)**.

### Flu season is here!

Getting a flu vaccination is part of the recommended comprehensive care for thalassemia.

Learn more about the flu at: [www.cdc.gov/flu/about/index.html](https://www.cdc.gov/flu/about/index.html)

Please check with your doctor for further information.

This message was developed as part of Cooperative Agreement #6NU27DD001150-05-01 from the Centers for Disease Control and Prevention.

Cooley's Anemia FOUNDATION  
Leading the Fight Against Thalassemia  
[www.thalassemia.org](https://www.thalassemia.org)

## CDC PROJECTS INCLUDE THALASSEMIA WEBINARS AND GRAND ROUNDS

CAF has been fortunate to work on several cooperative agreements with the US Centers for Disease Control and Prevention (CDC). Part of the work that the Foundation does under the current agreement involves webinars and "grand rounds" presentations that focus on thalassemia.

CDC's Division of Blood Disorders presents a number of webinars each year in its Public Health Webinar Series on Blood Disorders. The purpose of this series is to provide evidence-based information on new research, interventions, emerging issues of interest in blood disorders, as well as innovative approaches in collaborations and partnerships. Several of these webinars have focused on thalassemia, most recently on June 24, 2021 when Dr. Alexis Thompson presented on "Bone Marrow Transplantation and Other Curative Approaches in Thalassemia." (All of the webinars in this series can be accessed at <https://www.cdc.gov/ncbddd/blooddisorders/webinar.html>.) For the thalassemia-focused webinars, CAF provides assistance to the Division of Blood Disorders to plan and promote them. The next thalassemia webinar will be held November 18, 2021 and will focus on "Cardiovascular Issues and Management in Thalassemia."

As part of the current cooperative agreement, CAF is also working with CDC to create 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. The first presentation in this series was held January 29, 2021; Dr. Mona Al Mukaddam presented on "Evaluating and Managing Bone Health in Thalassemia Patients." (The presentation can be accessed at <https://www.cdc.gov/ncbddd/thalassemia/webinar-archives.html>.) The next Grand Rounds, the topic of which is "The Non-Transfusion-Dependent Thalassemias: An Enduring Challenge," is scheduled for October 29, 2021.

CAF thanks CDC for its commitment to thalassemia and its commitment to creating these important educational resources and making them available to healthcare providers in general and the thalassemia community in particular.

### In Memoriam

We regretfully report the loss of Cooley's anemia patients

**Sujit Bahra, Michelle Basso, Victoria Chui, Meghan Foe, Lisa Falco Giudice, William Matrisciano, Josephine Micco, Ethan Tran and Titus Wilhite.**

## CAMPS FOR CHILDREN WITH THALASSEMIA

CAF is happy to provide information about camps for children with serious medical conditions including thalassemia. This is provided for informational purposes, and CAF does not endorse one camp over another. We recommend visiting the camp websites for more information about camp sessions, planned activities, dates, amenities, and application instructions.

- THE PAINTED TURTLE – LAKE HUGHES, CA**  
Oct 2-3, 2021: Fall Overnight Family Retreat  
[www.thepaintedturtle.org](https://www.thepaintedturtle.org)
- THE DOUBLE H RANCH – LAKE LUZERNE, NY**  
[www.doublehbranch.org](https://www.doublehbranch.org)
- CAMP KOREY – MT. VERNON, WA**  
[www.campkorey.org](https://www.campkorey.org)
- HOLE IN THE WALL GANG CAMP – ASHFORD, CT**  
[www.holeinthewallgang.org](https://www.holeinthewallgang.org)
- ROUNDUP RIVER RANCH – GYPSUM, CO**  
Sept 2-30, 2021 (Thursdays): Online Art Club  
Oct 4-26, 2021 (Tuesdays): Online Discovery Club  
Oct 8-10, 2021: Online Family Camp  
[www.roundupriverranch.org](https://www.roundupriverranch.org)
- VICTORY JUNCTION – RANDLEMAN, NC**  
Oct 29-31, 2021: Family Retreat  
[www.victoryjunction.org](https://www.victoryjunction.org)
- CAMP CAREFREE – STOKESDALE, NC**  
[www.campcarefree.org](https://www.campcarefree.org)
- NORTH STAR REACH – PINCKNEY, MI**  
September 24-26, 2021: Sickle Cell/Hematology Camp  
[www.northstarreach.org](https://www.northstarreach.org)
- CAMP HOLIDAY TRAILS – CHARLOTTESVILLE, VA**  
[www.campholidaytrails.org](https://www.campholidaytrails.org)
- CAMP BOGGY CREEK – EUSTIS, FL**  
Sept-Dec, 2021 – Various Camps  
[www.boggycreek.org](https://www.boggycreek.org)
- FLYING HORSE FARMS – MT. GILEAD, OH**  
Oct-Nov, 2021 – Various Camps  
[www.flyinghorsefarms.org](https://www.flyinghorsefarms.org)



# UPDATES ON NOVEL TREATMENTS IN THALASSEMIA

There are currently several novel therapies in thalassemia on the horizon. Below are updates on clinical trials in gene therapy and gene editing. We are also sharing information about the BELIEVE Study for Luspatercept (Reblozyl), which is a novel treatment for reducing iron levels in thalassemia. Visit [thalassemia.org](https://thalassemia.org) to stay up to date with the most recent developments.

## LUSPATERCEPT (REBLOZYL)

The effect of long-term luspatercept use on iron levels and on utilization of iron chelation therapy was assessed in the Phase 3 BELIEVE trial of luspatercept in adult patients with transfusion-dependent beta thalassemia.

After 24 weeks of treatment, 17.0% of patients who took luspatercept showed a reduction in serum ferritin levels from  $\geq 1,000$   $\mu\text{g/L}$  to below 1,000  $\mu\text{g/L}$ , compared with only 5.0% of patients treated with placebo.

After 48 weeks of treatment, 9.7% of patients taking luspatercept showed a reduction in Liver Iron Concentration (LIC) from  $>3$  at baseline to  $\leq 3$  mg/g dry weight, compared with only 5.9% of patients treated with placebo. Also after 48 weeks of treatment, 20% of patients taking luspatercept showed an improvement in cardiac iron T2\* from  $\leq 20$  ms at baseline to  $> 20$  ms, compared with only 9.1% of patients treated with placebo.

No significant difference in iron chelation therapy was observed between luspatercept- and placebo-treated patients during the first 48 weeks of the study. However, the proportion of patients taking 1 or more iron chelation medications gradually declined in luspatercept-treated patients relative to patients taking placebo over longer time periods. Luspatercept-treated patients also experienced a gradual decrease in mean daily dose of deferasirox over time.

In summary, a higher percentage of luspatercept-treated patients compared to placebo-treated patients experienced reductions in serum ferritin levels, Liver Iron Concentration, and cardiac iron levels in the first 48 weeks of the BELIEVE Study. This suggests that luspatercept-treated patients may be at lower risk of the complications associated with iron overload. Long-term luspatercept treatment also led to decreasing trends of iron chelation therapy use and deferasirox dosage.

## GENE THERAPY

### Lentiglobin

Initial results from Phase 3 clinical trials showed that 10/12 adult patients with transfusion-dependent beta thalassemia achieved transfusion independence following treatment with bluebird bio's beti-cel (LentiGlobin) gene therapy for  $\beta$ -thalassemia. After showing initial success in adult patients, these trials were expanded to include adolescents and children. Preliminary findings from the pediatric trials of beti-cel in beta thalassemia were presented by Dr. Alexis Thompson of the Ann & Robert H. Lurie Children's Hospital of Chicago, IL at the 2020 American Society of Hematology (ASH) annual meeting.

24 pediatric patients, ranging from 4 to 17 years of age, were treated with beti-cel gene therapy. 14 of these patients were enrolled in the study of beti-cel in patients with non-B0/B0 genotypes. Of these, 10 patients were ready for evaluation at the time the analysis was performed. Transfusion independence is defined as a weighted average hemoglobin  $\geq 9$  g/dL without transfusions for at least 1 year. In this dataset, transfusion independence was achieved in 3/4 (75% of) patients under 12 years of age, with a weighted average hemoglobin of 10.0 g/dL. In contrast, 6/6 (100% of) patients ages  $\geq 12$  to  $<18$  years of age achieved transfusion independence, with a weighted average hemoglobin of 11.6 g/dL.

10 pediatric patients were enrolled in the study of beti-cel in patients with the more severe B0/B0, B0/B+IVS-1-110, or B+IVS-1-110/ B+IVS-1-110 genotypes. Of these, 5 patients were ready for evaluation at the time the analysis was performed. In this dataset, transfusion independence was achieved in 1/2 (50% of) patients under 12 years of age, with a weighted average hemoglobin of 10.3 g/dL. In contrast, 3/3 (100% of) patients ages  $\geq 12$  to  $<18$  years of age achieved transfusion independence, with a weighted average hemoglobin of 9.6 g/dL.

Grade 4 veno-occlusive disease occurred in 2 patients and grade 2 veno-occlusive disease occurred in 1 patient; all cases were successfully treated with defibrotide. The safety profile of gene therapy with beti-cel in this pediatric population was consistent with the side effects typically observed with busulfan conditioning.

Preliminary findings from these studies indicate that pediatric patients  $< 18$  years of age achieved transfusion independence at comparable rates to adult patients, suggesting that beti-cel gene therapy represents an effective treatment option across age groups for patients with transfusion-dependent beta thalassemia.

### GENE EDITING

#### ST-400

Sangamo Therapeutics/Sanofi have announced preliminary findings from the first 3 beta thalassemia patients treated with their ST-400 gene editing therapy in the Phase 1/2 THALES Study. ST-400 is a therapy that involves gene editing of a patient's own hematopoietic stem cells (HSCs) in order to reactivate fetal hemoglobin production. The first treated patient, who

has a  $\beta 0/\beta 0$  genotype, initially showed increased fetal hemoglobin levels and became transfusion-independent for a period of 6 weeks. However, after an initial transfusion-free period, this patient subsequently resumed intermittent transfusions. The second treated patient, who is homozygous for the severe  $\beta +$  IVS-I-5 (G $>$ C) mutation, showed increased fetal hemoglobin levels relative to baseline. However, this patient was still receiving intermittent transfusions, 26 weeks after treatment. The third treated patient, who has a  $\beta 0/\beta +$  genotype, initially experienced a transfusion-free period of 7 weeks. However, this patient also subsequently resumed intermittent transfusions following the initial transfusion-free period.

#### CTX-001

CRISPR Therapeutics and Vertex Pharmaceuticals have announced early results from their CLIMB-111 Study in which transfusion-dependent beta thalassemia patients are treated with a gene editing therapy called CTX001. CTX001 is a therapy in which CRISPR/Cas9-based gene editing is performed on a patient's hematopoietic stem cells in order to reactivate fetal hemoglobin production. The results of the first 15 patients treated in the study, who have achieved at least 3 months of follow-up, are now available: All 15 patients are now transfusion independent, with follow-up ranging from 4 to 26 months after CTX001 infusion. All 15 patients showed clinically meaningful improvements in their total hemoglobin levels (from 8.9 to 16.9 g/dL) and fetal hemoglobin levels (from 67.3% to 99.6%) at last visit. This includes six patients with the beta zero/beta zero or other severe genotypes.

The safety data from all 15 patients are consistent with what is typically observed following autologous stem cell transplant and myeloablative conditioning. As previously reported, one patient experienced four serious adverse events considered related or possibly related to CTX001: headache, hemophagocytic lymphohistiocytosis (HLH), acute respiratory distress syndrome and idiopathic pneumonia syndrome. All four serious adverse events occurred in the context of HLH and have resolved. The majority of non-serious adverse events were considered mild to moderate.

## CAF CHOSEN AS PART OF HRSA THALASSEMIA PROGRAM

The Cooley's Anemia Foundation has been awarded a cooperative agreement with the Health Resources and Services Administration (HRSA) as part of their 5-year Thalassemia Program project. The Foundation will be working with awardees from Ann & Robert Lurie Children's Hospital Chicago; Children's Hospital of Philadelphia; New York-Presbyterian Phyllis and David Komansky Children's Hospital and Weill Cornell Medical Center; and UCSF Benioff Children's Hospital Oakland, as well as with staff from HRSA.

The goal of this program is to improve access to evidence-informed care for individuals with thalassemia. As part of this Program, the hospitals chosen for the award

## CAF ESTABLISHES ENDOWMENT FUND

The Cooley's Anemia Foundation has established an Endowment to provide a long-term, secure funding stream for CAF programs. Monies donated to the Endowment are set aside in an investment fund whose principle remains intact and whose gains are made available for program use. This investment fund is guided by an Investment Policy (available upon request), approved by the Board of Directors, and overseen by the Investment Committee of the Board. The Investment Policy details the establishment and oversight of the Endowment investment portfolio and the conditions for withdrawing funds.

The Endowment is funded through the generosity of donors. All monies will be used to fund the Endowment, and the proceeds from the Endowment will be used to fund Cooley's Anemia Foundation programs.

CAF has already received several donations to the Endowment, and we gratefully acknowledge the generous contributions to the Endowment from the following:

- The Leo Arvanitakis Family
- Jeanne Bassani Miller
- The Chieco Family
- Michael DiFilippo and Family
- The Goel Family
- The Pahuja Family

**TO LEARN MORE ABOUT OR MAKE A CONTRIBUTION TO THE ENDOWMENT, PLEASE CONTACT CRAIG BUTLER, [CBUTLER@THALASSEMIA.ORG](mailto:CBUTLER@THALASSEMIA.ORG).**

## FOUR WINNERS SELECTED FOR NEW CAF-ACCELERON SCHOLARSHIPS

The Cooley’s Anemia Foundation is proud to announce the four winners of the inaugural CAF-Acceleron Scholarships: Katrina Wagar, Loula Chen, Hetvi Patel, and Kallie Shanahan. These awards are each in the amount of \$5,000 and are presented to individuals in the United States with a clinically significant form of thalassemia (alpha or beta thalassemia major, beta thalassemia intermedia, e beta thalassemia, hemoglobin H disease or hemoglobin H disease Constant Spring). These scholarships are made possible through a grant from pharmaceutical manufacturer Acceleron to encourage and support the pursuit of higher education by members of the thalassemia patient community.

*“We’re thrilled to join the Cooley’s Anemia Foundation in awarding scholarships to such deserving recipients. It’s a privilege to support these students in pursuit of their academic goals and help prepare them to become future leaders, ambassadors, and advocates within and for the thalassemia community.”*

– Todd James, Senior Vice President of Corporate Affairs, Acceleron

These awards are merit-based and place significant emphasis on the applicants’ academic records and quality of the personal essays. Other factors include the strength of the letters of recommendation, as well as involvement in the thalassemia community. Three of scholarship recipients (Katrina Wagar, Loula Chen, and Hetvi Patel) are currently enrolled in a college program and will continue to be enrolled in a college program in the upcoming fall semester. One of the awardees, Kallie Shanahan, just finished high school and will be starting a college program in the fall.

“I am sincerely honored to have been selected as a recipient of the Acceleron scholarship,” said Katriana Wagar. “Thank you to Acceleron and the Cooley’s Anemia Foundation for your generosity. As I work to complete my masters in Physician Assistant at D’Youville College this year, this scholarship will help tremendously with tuition. I am very grateful to be apart of an organization that supports it’s members and patients as well as values the importance of education. Your generosity is allowing me fulfill to my dream of helping others like me in the field of hematology as a Physician Assistant.”

**We congratulate the winners of these awards and thank Acceleron for supporting our patients by funding this scholarship program.**

## CAF NEEDS YOUR HELP

As our longtime supporters know, CAF prides itself on being a small foundation which does big things. From putting on important Patient-Family Conferences every year to helping patients travel to treatment centers for annual comprehensive care evaluations to funding medical research that lays the groundwork for breakthroughs in treatment to making sure that government agencies know of the issues of importance to the thalassemia community, CAF is constantly working to improve the lives of those touched by thalassemia. We do impressive work – but we need your help if we are to continue to fight effectively against thalassemia.

Like most non-profits, we suffered a big financial hit due to the COVID-19 pandemic. Approximately one-third of our funding comes from events run by our chapters – and once the pandemic struck, these events had to be cancelled. In addition, funding from individual donors fell as well.

But while our funding decreased, the needs we seek to address were not; if anything, they increased. We significantly increased the activities of our Patient Services department, especially those aspects overseen by our Social Worker, Kathleen Durst, as the pandemic raised many issues among our community that needed to be addressed. The pandemic also meant that we spent a great deal of time seeking out and distributing information about COVID-19 that was relevant to our community.

We are proud to have done so much over the last year and half, and we hope you see the value in all that CAF has done, both recently and historically. We hope that you can take a moment to help us continue our good work by making a donation today. You can fill out the form on page 15 or go to <https://app.etapestry.com/hosted/CooleysAnemiaFoundation/OnlineDonation.html> to make an online donation.

You have been there for us before, and we are so grateful for that. Please take a moment and show your continued support today.

Thank you,  
Craig Butler, National Executive Director

## CAF AWARDS 32 STUDENTS WITH INCENTIVE AWARDS TOTALING \$32,250

The Cooley’s Anemia Foundation is pleased to announce that 26 students with thalassemia and 6 children of individuals with thalassemia were awarded a total of \$32,250 in scholarship funding as recipients of the CAF Patient Incentive Awards. These awards are open to thalassemia patients and children of patients who are U.S. residents and are currently pursuing education in a graduate, undergraduate, associate, certificate or vocational level. The Patient Incentive Awards range from \$250 for those enrolled in six-month certificate programs to \$2,000 for students enrolled in MD, JD, or PhD programs.

While much of the CAF’s focus is on advancing medical research to find better treatment options and a cure, another important goal is to improve the everyday lives of patients in ways beyond medical treatment. The Foundation launched its Incentive Award Program in 1994 with the purpose of encouraging patients to further their education and career goals and to help them live positively with thalassemia. In 2019, CAF added the Educational Incentives for Children of Individuals with Thalassemia award category. Since the Incentive Program was launched in 1994, CAF has awarded over \$557,000 in awards to 502 deserving students (402 patients, and 10 children of patients) in pursuit of higher education.

CAF congratulates each of these remarkable students, and we wish them the very best as they pursue their careers. Below is a partial list of the 2019-2020 Patient Incentive Awards recipients.

- |                       |                        |
|-----------------------|------------------------|
| Juntra Chanthavisay   | Vincent Phan           |
| Dara Chanthavisay     | Nikki Phan             |
| Alexia DeBoe          | Grace Mei-Peng Richard |
| Arjun Dhar            | Hanna Richards         |
| Shameem Fakory        | Vivian Tran            |
| Isabella Fremer       | Bryan Tran             |
| Patroula Galanoupolou | Linda Wong             |
| Sevda Gerger          | Sergio Joseph          |
| Nicholas Larkin       | Matthew Fasani         |
| Tobias Larkin         | Sophia Fasani          |
| Hamza Mahmood         | Stephen LaGreca        |
| Natalie Maino         | Hanna Lazio            |
| Aeshah Nadeem         | Taylor Lazio           |
| Amanda Nguyen         |                        |

## CAF INTRODUCES ANIMATED VIDEOS ON EMOTIONAL HEALTH

Under the direction of our social worker Kathleen Durst, CAF is pleased to share several new animated videos on emotional health. These videos cover topics such as managing thoughts and emotions, tolerating and sending away physical pain, taking care of yourself, and understanding and managing worry. To watch these videos, visit: [thalassemia.org/emotionalhealthvideos](https://thalassemia.org/emotionalhealthvideos).

CAF Social Worker Kathleen Durst says, “My goal for the videos was to help everyone to understand emotional health and that we are not alone. No one is immune to depression and anxiety. We have been greatly challenged due to Covid. So my hope is that people can walk away with skills they can use to do self-therapy and reduce the emotional burden themselves. However, if a higher level of care is needed, they can discuss with their PCP, hematologist, NP or contact me and I can help them find resources.”

U.S. thalassemia patients and family members should contact Kathleen at [kdurst@thalassemia.org](mailto:kdurst@thalassemia.org) if they wish to learn more about the work that Kathleen does with members of our U.S. thalassemia community. A special thank you goes to Bristol Myers Squibb for providing funding that supports the creation of these important videos.





PATIENT PROFILE: NOOR ALTAHAFEE

“Thalassemia, and the challenges that accompany it, have helped me become stronger and overcome many difficulties and negative feelings.” This is the perspective of Noor Altahafee, a young woman who, throughout her life, has coped with the demands of thalassemia, an inherited blood disorder in which the red blood cells aren’t able to get enough oxygen to the tissues and organs in the body. Noor was born in Mosul, Iraq, and started receiving regular red blood cell transfusions at 5 years of age in order to treat her thalassemia.

Living in Iraq made it difficult for Noor to obtain quality thalassemia care. The blood bank in Mosul often did not have access to blood testing kits to ensure that donated blood was free of infectious agents such as HIV (human immunodeficiency virus) or the hepatitis B or C viruses. If, when arriving at the hospital for treatment, the blood that was available had not been tested for viruses, Noor’s mother would refuse treatment to prevent Noor from possibly acquiring an infectious disease. Noor would then have to wait and later return to the hospital when blood had been thoroughly tested and deemed safe.

Another serious problem for Noor and other people living with thalassemia in Iraq was that few people had access to chelation medicine, which removes excess iron from the body. This is important, because one side effect of having frequent transfusions is iron overload. If left untreated, the excess iron in the body can lead to complications such as heart failure or liver cirrhosis, a condition in which the liver is scarred and permanently damaged. On rare occasions, the chelation medicine called deferoxamine would be available at the local hospital in Mosul. However, if the medicine was not available, Noor’s mother often had to purchase it through friends who lived in other countries such as Jordan or Syria. Likewise, it was difficult to obtain the sterile water needed to rehydrate powdered deferoxamine, as well as the needles and syringes needed for administration, because the supply of these materials was so limited. These products were also purchased through friends in other countries such as Jordan or Syria, when necessary. Although the medication was expensive, Noor’s family made the sacrifice to get it because they understood that chelation was essential for Noor’s long-term health.

Noor was 10 years old when war broke out in Iraq in 2003. The ongoing bombings and the presence of military checkpoints made it difficult for Noor to attend school and for her father to operate his wholesale cosmetics store. Noor’s mother worked as the Director of Research in the Department of Computer Science at Mosul University, but became increasingly frightened to go to work because of the ongoing war. Noor’s family remained in Mosul while the country was at war for the next 5 years. However, the war made it more difficult than ever for Noor to obtain the red blood cell transfusions and chelation medicine that her life depended on.

Because there were very few resources available to support people with thalassemia living in Iraq, Noor’s mother, Faten



Alabbas, helped to establish the Thalassemia Association of Niniva (TAN), based in Mosul, Iraq. Faten served as vice president of TAN for 13 years, and was able, through a partnership with a group in Italy, to establish a thalassemia center in Mosul that included a bone marrow transplant center. Faten hoped that Noor might be cured of thalassemia through a bone marrow transplant. Unfortunately, no one in her family was a close bone marrow match. Faten then reached out to a center in Sardinia, Italy, in search of an unrelated bone marrow donor for Noor. As luck would have it, they identified an individual in the United States who was a 100% match for Noor and who was willing to donate bone marrow. Unfortunately, this potential donor was involved in a car accident shortly thereafter that left him unable to follow through with the donation. When Faten broke the news to Noor, she said, “Do not be sad or upset that this fell through. It is a sign from God. Our plan now will be to focus on providing you with the best possible thalassemia treatment we can find. Who knows what the future holds in store for you.”

Noor and her mother left Iraq in 2014 shortly after a military offensive in Mosul heavily damaged the entire city, including its hospitals and clinics. Many homes were burned to the ground, including Noor’s grandparents’ home. Noor and Faten escaped by relocating to the Kingdom of Bahrain in the Arabian Gulf. They sought permanent residence status in Bahrain, but were denied. They then relocated with the rest of their family to Turkey in 2015 and applied for refugee status with the United Nations High Commissioner for Refugees (UNHCR). UNHCR helped Noor’s family move to the United States. The family moved to Massachusetts in 2016 to be near Iraqi relatives who had previously resettled there (including a younger cousin, Dema, who also has thalassemia and was receiving excellent care locally).

Noor is now 26 years old and extremely grateful to be receiving first-rate healthcare from the thalassemia center at Boston Children’s Hospital.

“As a person living with thalassemia, feeling that others are helping you and following up on your care gives you more energy to be a productive person.”

She is also enjoying the flexibility of the educational system in the United States. Noor already had an Associate’s Degree in

Business from Iraq. Since moving to the United States, she has decided to pursue her dream of working in the healthcare field instead and is now studying to become a dental hygienist.

Since relocating to the United States, Faten has remained in contact with TAN and people with thalassemia and their families in Iraq, providing them with up-to-date information about the latest advances in treatment. Noor has not returned to Iraq since leaving in 2014, but hopes to visit her homeland again someday. She worries about others with thalassemia still living in Iraq. “I hope that we can help them in some way and let them know that they are still on our minds.”

Noor is profoundly indebted to her family, and especially to her mother, for helping her overcome all the struggles she has faced. “After God, my mother is the source of my energy in this life.”

HERE ARE SOME OF NOOR’S TIPS FOR OTHERS LIVING WITH THALASSEMIA:

- Each day is a gift to be cherished.
- Be faithful, positive, and productive.
- Remember that there are other people whose health difficulties are even worse than yours.
- Strive to be your best, but also recognize that some things cannot be changed and so you will have to learn to accept them.

CDC and CAF thank Noor for sharing her personal story.  
This profile was developed as part of Cooperative Agreement #6NU27DD001150-05-01 from the Centers for Disease Control and Prevention.

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

2021 CAF PATIENT-FAMILY CONFERENCE WAS A HYBRID AFFAIR

After hosting an online-only mini-Conference in 2020, CAF offered patients and family members an opportunity to attend its 2021 Patient-Family Conference either in person or virtually. The Foundation is pleased to report that the hybrid event was a significant success.

Working within strict guidelines to minimize the chance of COVID-19 infection, CAF welcomed more than 175 patients and family members to the Hyatt New Brunswick in New Jersey. This was the first time in two years since that the Foundation was able to hold a live national event, and the joy felt by attendees was palpable. Though masked and socially distanced, participants had the pleasure of being face to face with other members of the community, and many remarked on what a difference the in person experience makes.

The sessions on Saturday were webcast live so that anyone not able to attend in person was able to hear the presentations, send in questions, and share the experience virtually. More than 100 people participated online. Topics ranged from “When to Transfuse” to “An Alpha Thalassemia Update” to “Iron and Aging in Thalassemia.” Eashani Ghosh, a thalassemia patient, presented on a study she had conducted on hydration and thalassemia.

Plans are underway for next year’s Conference, to be held in Chicago July 15-17, 2022. We hope you can join us!

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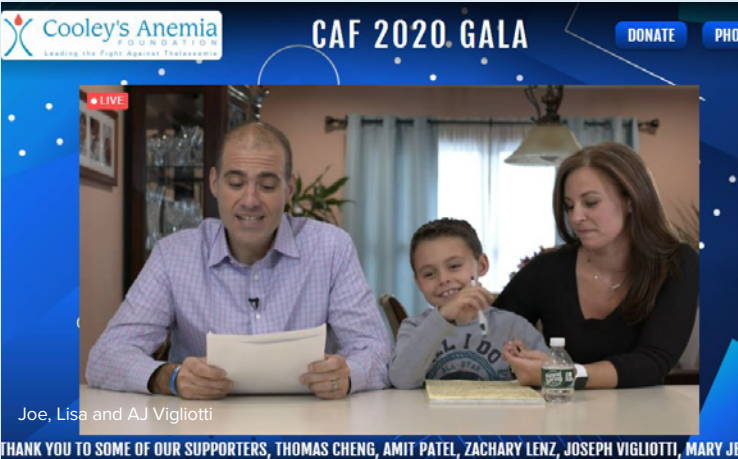
## CAF'S FIRST VIRTUAL GALA A NOTABLE SUCCESS [CONT.]

Chieco, National Volunteer President of the Cooley's Anemia Foundation. "He stood out as a beacon of light and helped us set a Gala fundraising record. We will always remember his strength and determination to see this Gala be successful."

"I can assure you that the cure is coming," said Mr. Scaramucci upon receiving his award. "And the reason it's coming is because of the foundational work we are doing on a night like this when we aren't actually together but we are together as one supporting the goal of curing Cooley's anemia. I want to thank the Foundation for honoring me. It's a great honor, but it's really about you. I want to thank all of you here for the donations that you're making to help us get to this cure. Thank you, again."

**WE THANK ANTHONY SCARAMUCCI FOR HIS SUPPORT OF THE FOUNDATION AND CONCERN FOR ALL THOSE WITH THALASSEMIA.**

This year, CAF is hosting a special online Evening of Giving on Tuesday, October 19 at 7:00 pm. There is no charge to attend this special event but reservations are required to receive the necessary link. More information at: [fundraising.thalassemia.org/cafgiving2021](https://fundraising.thalassemia.org/cafgiving2021)



## CARE WALK GOES VIRTUAL [CONT.]

we are able to offer patient incentive awards to encourage patients to seek higher educational goals; fund a new program to aid patients travel long distances to major treatment centers for comprehensive care services; hold our important Patient-Family Conferences which continue to grow at an amazing rate; support important medical research; and so much more.

We would like to take this opportunity to thank all of our volunteers for the incredible work they have done to grow this special event and make it the success it has become. All of the time and energy our volunteers invest into planning their Care Walks, soliciting donations, and educating the community about thalassemia has made Care Walk have the most impact of any single event of the year for the Foundation. CAF is enormously grateful to each of you for your hard work, dedication, and determination. We also want to thank each of our donors for all your support.

**REGISTER FOR CARE WALK 2021 TODAY!**

Some teams have already begun to host Care Walks this year, and others will host theirs in the upcoming months. The beauty of having Care Walk happen in many locations all over the country is that you can organize your own event and tailor it to your schedule and the specific needs of your community.

Whether or not you will be gathering with others to walk in person, we encourage you to join us for our second annual Care Walk by registering to participate (t-shirt included), making a donation, and tuning into the online stream of the virtual event.

**VISIT [BIT.LY/CAREWALK2021](https://bit.ly/carewalk2021) TO REGISTER, DONATE & WATCH THE VIRTUAL CARE WALK.**



THANK YOU TO OUR CARE WALK SPONSORS

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# LEGACY SOCIETY REGISTRATION FORM

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

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

- ☐ Last Will and Testament
- ☐ Gift of Real Estate
- ☐ Transfer on Death (TOD) on Account
- ☐ A Codicil in a Will
- ☐ Retirement Plan Beneficiary Designation
- ☐ Contribution to Endowment
- ☐ Individual Retirement Account Charitable Rollover
- ☐ Charitable Remainder Trust
- ☐ Other \_\_\_\_\_
- ☐ Charitable Gift Annuity
- ☐ Charitable Lead Trust
- ☐ 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 PARTNERS WITH FREEWILL TO PROVIDE A NO-COST LEGAL WILL

Planned Giving is for everyone, regardless of age. Whether you are just beginning to consider making a will or wanting to modify an existing will, CAF now has a way for you to do so at no cost to you! We have engaged the services of FreeWill, a web-based organization that can help you make a will that is legal in all fifty states. And you can use this tool whether or not you plan to leave a legacy gift to CAF and whether or not you wish to join our Legacy Society – it is simply our way of encouraging you make a will (which we highly suggest everyone does) at no cost to you.

Here’s how it works:

1. Visit www.FreeWill.com/CAF to create your legal will online or to document your wishes and find an attorney near you.
2. If you choose, you can specify within your will the amount of percentage of your estate you’d like to leave to Cooley’s Anemia Foundation and/or other charities.
3. You can now rest easy, knowing you have checked off making a will from your to-do list!

Remember that there are no strings attached to this service.

We want you to have access to this opportunity because we believe that everyone should have a will. If you choose to include CAF in your legacy plans, we are very appreciative, but it is not a condition for using FreeWill.

Whether you use FreeWill or use the services of an attorney directly, and choose to leave a gift to CAF, the following language is suggested:

“I/We bequeath (amount OR percentage) to Cooley’s Anemia Foundation, a nonprofit corporation organized and existing under the laws of New York, with the principal business address of 330 7TH Ave Ste. 200 New York, NY 10001-5279 and federal tax identification number 11-1971539.”

If you have any further questions, please contact:  
Cooley’s Anemia Foundation, 330 Seventh Avenue, Suite 200, New York, NY 10001  
Telephone: (212) 279-8090 ext. 201  
Email: info@thalassemia.org

✂

# 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 \$\_\_\_\_\_

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ADDRESS\_\_\_\_\_

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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.



## CARE WALK GOES VIRTUAL [SEE STORY ON COVER]



Go Green! Email [mary@thalassemia.org](mailto:mary@thalassemia.org) to receive future issues of Lifeline via email.

*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.*

