



WINTER 2018

LIFELINE

COOLEY'S ANEMIA FOUNDATION • LEADING THE FIGHT AGAINST THALASSEMIA

RECORD-BREAKING ATTENDANCE AT THE CAF 2018 PATIENT-FAMILY CONFERENCE

The 2018 CAF Patient-Family Conference was a true success, with a record-breaking attendance of over 400 members of the U.S. thalassemia community. This annual Conference provides up-to-date information on thalassemia treatment, and gives members of the thalassemia community an opportunity to get to know each other and share experiences.

The Conference featured a talk by Janet Kwiatkowski, Chair of the CAF Medical Advisory Board, who provided an update on new therapies in development for thalassemia. Other speak-

ers presented information on fertility and pregnancy, cardiac issues, nutrition, endocrine issues, and chelation. In addition to medical updates, speakers shared information about transitioning into adult care, managing insurance, and living positively with thalassemia. The Conference attendees also had the opportunity to participate in group breakout sessions focused on parents, children, teens, adult patients, patients who are also parents, and friends, spouses and partners.

A major highlight for many was the Saturday night dinner dance, where everyone had a chance to mingle and let loose on the dance floor. A big hit at this year's dinner dance were the caricature artists who made hilarious illustrations of the party-goers, as well as balloon artists who delighted the children by twisting balloons into animal shapes. Above all, members of the thalassemia community had an invaluable opportunity to get to know one another better, make new friends, and connect with old friends. We thank everyone who attended for making the Conference so very special this year, and we hope to see even more of you next year!



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CAF LAUNCHES THREE NEW PATIENT SERVICES PROGRAMS

The Cooley's Anemia Foundation is proud to announce three new programs intended to benefit members of the U.S. thalassemia community. These programs are:

SUPPORT FOR SIGNIFICANT TRAVEL TO TREATMENT CENTERS

The Cooley's Anemia Foundation (CAF) is offering limited financial assistance to individuals with thalassemia who live 150 or more miles from a major thalassemia treatment center and who experience financial hardship due to travel expenses incurred in obtaining an annual comprehensive care evaluation or extraordinary thalassemia care at a major treatment center. (Extraordinary thalassemia care shall be defined as care recommended by a qualified thalassemia physician which falls outside regular transfusions or comprehensive care – for example, if a patient requires multiple MRI iron assessments which cannot be performed at their home treatment facility.)

CAF will reimburse up to \$500 in travel expenses to qualified applicants. Reimbursements will be distributed on a first come, first serve basis until funding for the period has been exhausted. No one thalassemia patient may receive more than \$500 travel reimbursement on an annual basis under the terms of this program. Families with more than one child with thalassemia may be reimbursed for up to \$500 for each child. Travel must have occurred on or after July 1, 2018.

Because funds are limited, patients are encouraged to check with CAF in advance if they are planning on utilizing this program for reimbursement. CAF will make every effort to assist qualified applicants but cannot provide funding once it is exhausted for the year. Total funding for the initial year is \$30,000.

SUPPORT FOR PARENTS OF NEWLY ADOPTED CHILDREN WITH THALASSEMIA

The Cooley's Anemia Foundation (CAF) will award \$1,000 to parents of newly-adopted children with significant forms of thalassemia in recognition of their willingness to provide a new home to these important members of the thalassemia community. (A significant form of thalassemia would include thalassemia major, thalassemia intermedia, e beta thalassemia, hemoglobin H disease or hemoglobin H constant spring.) This will apply to children adopted on or after July 1, 2018.

Support will be dependent upon availability of funding. In the event that the number of applicants exceeds the amount of funding available, the Foundation reserves the right to make adjustments as necessary in the number of awards given and/or the level of funding for each applicant.

EDUCATIONAL INCENTIVE AWARD PROGRAM FOR CHILDREN OF INDIVIDUALS WITH THALASSEMIA

The Cooley's Anemia Foundation is initiating an educational incentive award program for children of U.S. individuals with severe thalassemia to further their education and career goals. These awards will be made this year in March, 2019 for students enrolled in the fall 2018 and/or spring 2019 semester(s). The total amount of funding for this program for the current 2018-2019 school year is \$10,000.

It is the goal of The Cooley's Anemia Foundation to fund all complete, eligible applications received by February 15, 2019. However, in the event that the number of applicants exceeds the amount of funding available for these Incentive Awards, the Foundation reserves the right to make adjustments as necessary in the number of awards given and/or the level of funding for each successful applicant.

The incentive awards will be given out as follows:

- \$1,000 will be awarded to students enrolled in Baccalaureate, Master or Doctorate programs. Patients may receive four undergraduate, two master and two doctorate level awards over the course of their studies.
- \$500 will be awarded to students entering or continuing full time studies towards a two-year Associate degree, one-year certificate program or 6-month certificate or vocational training program. These are limited to two per applicant over the course of his/her pursuit of a two-year Associates degree, or one per applicant for a one-year or 6-month certificate or vocational training program.

For more information and to apply, email Eileen Scott at escott@thalassemia.org.

Exercise is Important for Optimal Health in People with Thalassemia

Exercise provides a number of important physical benefits including increased bone strength, increased muscle strength, and increased energy and stamina. It also offers mental health benefits such as reduced anxiety and depression, improved sleep, and improved mood.

Even a little moderate exercise every day may help to reduce the long-term risk of fracture or osteoporosis in people with thalassemia. Regular exercise may also help to increase your overall physical and emotional health.

Work with your doctor to come up with a physical activity plan that matches your abilities – especially if you have a history of pain, osteoporosis, or fracture – to reduce the risk of injury.

This message was developed as part of Cooperative Agreement #5N027DD001150404-00 from the Centers for Disease Control and Prevention.

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

INTRODUCTION LETTER FROM NEW COOLEY'S ANEMIA FOUNDATION PRESIDENT, PETER CHIECO



It is an indeed an honor and privilege to serve CAF again as National President. I would like to start by acknowledging and thanking my immediate predecessor as President, Anthony Viola, his wife Susan, and their family for the incredible success they achieved during Tony's ten years as President! Their sacrifice was great, and we all benefited from the results. We are eternally grateful for all their efforts.

Back in 1994, when I was 33, I was honored to be elected to this position, and despite the big time commitment, it was a great experience. Though, we had many challenges to overcome back then. Some we achieved, such as the creation of the Thalassemia Clinical Research Network. Others we laid the groundwork for, such as MRIs for heart and liver iron and the eventual approval of Ferriprox.

The additional benefit was our family grew up with a positive experience in living with the disorder, and the Foundation helped foster that attitude. Our middle daughter Michelle is a great example of how important the right outlook is in living successfully with thalassemia. We are so proud of her and her siblings for their commitment to make a difference in the lives of others.

I asked to serve as National President again for several reasons: It felt like the right time for my family and me, the Foundation has been one of the best blessings in our life, and the need is still great here in the U.S. and especially so in developing countries.

My hope is that we can increase the size of the Foundation's impact for all patients. At our board meeting on May 19th, we passed several new financial assistance programs to help serve patients and their families better. They are: Annual visit support to centers of excellence, stipends for adoptions of thalassemic children, and higher education support for children of thalassemia patients.

These programs, in conjunction with our current broad based activities, and the others to follow, highlight the enhanced ways the foundation can continue to lead in this global battle.

Of course, all of our activities require donations, and we are very focused on creating different ways donors can express their support for us. Currently, we primarily raise funds through our chapter events, national Gala, Care Walks (If you haven't done so, please join or start a walk for us next year), and general donations.

In fact, our recent national Gala was a great success on June 7th. A special thank you to Tracy Antonelli and her husband Patrick and their three incredible daughters for their amazing presentation as one of the night's honorees. They stole the night and set the tone for a great event!

We have also added two new fundraising programs that

have the potential to provide vital long term support to the Foundation. The first is a formal legacy society to recognize individuals and families that plan to bequeath us funds through their will, retirement accounts, insurance policies, and other estate bequests. We also are finalizing the creation of a permanent endowment fund to secure a stable source of funding and honoring in perpetuity the gifts of substantial donors.

I ask everyone to consider ways they can help the Foundation financially, either directly or through outreach to others. Please contact Henry Ackerman at the national office (212-279-8090 x 204 or hackermann@thalassemia.org) for assistance.

It is a very exciting time in the history of this disorder and yet challenges still remain in increasing the quality of life for all. Many patients struggle with complications from the impact of severe anemia, blood transfusions, and chelation therapy over time. Problems with normal growth, endocrine function, fertility, bone density, and blood safety and reactions all remain, and I have not listed all the potential complications. Longer lives have also created challenges with transition to adult care from pediatric settings, a great achievement, but one that can be problematic and stressful.

One easy way to help is by following us on social media, and for all U.S. patients to register with the foundation to receive benefits. Identifying every patient is crucial for us to help deliver all the benefits we have achieved. Please help us expand our reach by encouraging all patients to join our community.

In developing countries, the struggle to just survive is very much a present danger and one we must all look to change for the better. In some parts of China & India in particular, children and families are suffering, with little hope. We all have a shared responsibility to find ways to bring our success to all patients.

You have our word that we will work diligently and act thoughtfully to try and overcome every issue we face, and to invest the funds raised wisely, and for the benefit of the afflicted.

Our goal is simple: every patient, anywhere, deserves the opportunity to live their fullest life possible.

I thank you deeply for all the support you have given us over the years, and hope we can find ways together to continue the great tradition CAF has brought to the thalassemic community.

God bless, and with love,
Peter

Ps: The truth I learned over the years volunteering, which still holds true to this day, is that you receive so much more in return for your efforts to help.

CAF AWARDS \$227,500 FUNDING IN MEDICAL RESEARCH FELLOWSHIPS

CAF is pleased to announce that five new Cooley's Anemia Foundation Medical Research Fellowships and two renewal Fellowships have been awarded for the 2018-2019 grant cycle. The total amount of funding for the 7 research Fellowships is \$227,500.

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 Cooley's anemia. The CAF Grant 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 Grant Review Committee and Medical Advisory Board, and the other members of the Grant Review Committee for carefully reviewing the grant applications and making these important recommendations. We are excited to see what the investigators learn in these important and cutting-edge experiments.

FIRST-YEAR FELLOWS



Annamaria Aprile, PhD, of the Fondazione Centro in San Raffaele, Italy will examine hematopoietic stem cells (HSC) and their vascular niches in mice that exhibit properties typical of human beta-thalassemia. Her preliminary studies indicate that HSC in these thalassemic mice fail to self-renew and that this might be the result of a defect in the progenitors in the niche. Competitive transplants, including serial transplants, using thalassemic vs wild type HSC will reveal whether or not HSC exhaustion is autonomous or is due to the signals they are receiving from their surrounding damaged environment in the niche. These experiments have the potential to be extremely revealing of the underlying mechanisms that influence HSC and early progenitor activity in thalassemia, and why this altered marrow leads to ineffective red cell production.

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Chia-Yu Wang, PhD, of Massachusetts General Hospital will continue her work to identify the exact mechanism by which erythropoietin exerts its effect on iron regulation. When iron overload is present in patients with thalassemia, the increased red cell production in the bone marrow triggers the release

of erythropoietin, which signals the cells in the bone marrow and elsewhere to absorb and release even more iron. Identification of the mechanism by which erythropoietin signals its effect has the potential to lead to a new therapeutic approach to the management of iron overload in thalassemia.



Divya Vinjamur, PhD, of Boston Children's Hospital will investigate the role of the microtargetome, in particular the let-7 family of microRNAs (miRNAs), in fetal hemoglobin repression in adult erythroid cells. Identification of let-7 target genes that are effectors of HbF repression is a new approach that will likely identify a new set of proteins whose

globin-regulating activities can be therapeutically modified. Elevation of fetal hemoglobin in adult erythrocytes as a therapeutic strategy for beta-thalassemia is a promising area of research.



Raman Bahal, PhD, of the University of Connecticut, will test an innovative approach to gene therapy by injecting thalassemic mice with nano-particles containing advanced molecules which allow short segments of DNA to enter and fix thalassemia mutations. Dr. Bahal previously injected such nano-particles into thalassemic mice, resulting

in improved anemia and smaller spleens. In continuation of this work, Dr. Bahal will test an improved set of molecules in order to have an even greater impact. If successful, this could lead to thalassemia being cured by a series of injections without any intense chemotherapy to wipe out a patient's marrow.



Yvette Yien, PhD, of University of Delaware will be leading a study to better define key pathways leading the heme synthesis in red blood cell development. Dr. Yien's research group will utilize an innovative combination of iron/heme biochemistry, erythroid and yeast culture and mouse and zebrafish animal models to identify the detailed mechanisms

of iron transport. Knowledge gained through this study will be especially important in cases when red blood cell development is abnormal, as is the case with thalassemia.

SECOND-YEAR RENEWAL FELLOWS



Antonella Nai, PhD, of San Raffaele University in Milan, Italy will continue her study focused on understanding the role of TfR2 in red cell production and control of iron in thalassemia. Dr. Nai has shown that inhibiting TfR2 activity in thalassemic mice significantly improves anemia, ineffective red cell production, and iron-overload in a mouse

model of thalassemia intermedia. This year, she will test the effect of TfR2-antisense oligonucleotides as selective inhibitors of TfR2 to propose as a novel therapeutic agent for beta-thalassemia intermedia. She will also generate and analyze a model of thalassemia major with red-cell specific deletion of TfR2 in order to verify whether inactivation of TfR2 may be beneficial in the most severe form of the disorder.



Lei Yu, PhD, of University of Michigan Medical School will be continuing his study focusing on development of a small molecule analog to inhibit the activity of a repressor complex of fetal globin expression. Over the past year, Dr. Yu has made substantial progress by identifying a promising molecule. This year, he will modify the molecule to further

improve efficacy and will continue to investigate the mechanism of gamma globin induction. This study will bring this molecule closer to clinical application and thus merits the efforts to optimize its properties. Appropriate, in-house collaborations on pharmacology and efficacy have also been set up and thus increase confidence in the ultimate success of the approach.

REQUEST FROM CAF CHANGES LABEL

In September 2015, CAF and Sarah Baqueri-Connolly, the mother of a thalassemia patient who had recently passed away, appeared before a meeting of the FDA's Pediatric Advisory Committee. The Committee was launching a scheduled safety review of the iron chelator Exjade, and CAF and Sarah requested that the Committee investigate whether a label change was needed for the medication. When Sarah's daughter Zayna passed away, there was concern that use of Exjade should have been discontinued while a fever was present. The CAF Medical Advisory Board recommends discontinuation during a febrile illness, and the Foundation asked the Committee to consider recommending such a change to the label.

On September 20, the Committee heard the results of a thorough investigation into this issue. As a result of the request, and of Sarah and her husband Michael's willingness to share information with the investigating team, the label for Exjade and Jadenu has been altered to change the manner in which significant markers are measured and to adjust dosing in situations in which dosing at the current level might increase the risk of liver or kidney issues.

CAF thanks the members of the FDA Pediatric Advisory Committee and the investigative research teams for their thorough response to our request. And we especially thank Sarah and Michael for their commitment and dedication and for allowing access to the information that the team needed to arrive at their recommendations.

ACCEPTING APPLICATIONS: '18-'19 PATIENT INCENTIVE AWARDS

CAF is accepting applications for its 2018-2019 Patient Incentive Awards. These awards are open to thalassemia patients who are U.S. residents and are currently pursuing education in a graduate, undergraduate, associate, certificate or vocational level. The purpose of these Awards is to inspire patients to further their education and career goals and to help them live positively with thalassemia. We encourage all eligible US thalassemia patients to apply.

The deadline to apply is February 15, 2019.

Download the application here:
<http://bit.ly/CAF2019PIA>

SAVE the DATE

**Cooley's Anemia Foundation
Patient-Family Conference
July 12-14, 2019
San Francisco, CA**

Contact sgilbert@thalassemia.org to receive additional information when it becomes available.

VOLUNTEER SPOTLIGHT: MEET MICHELLE CHIECO: PRESIDENT OF CAF'S WESTCHESTER CHAPTER, REGISTERED NURSE, AND THALASSEMIA PATIENT

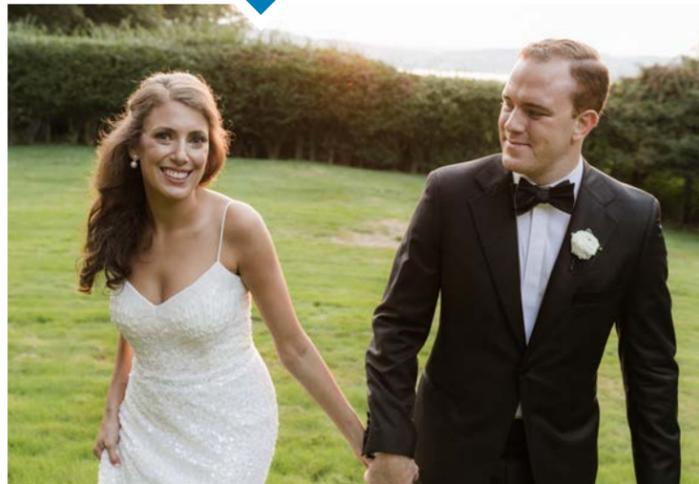
For over a year now, Michelle Chieco has been leading the CAF Westchester Chapter as Volunteer President. We thank her for her service, and for sharing her story with the community.

My name is Michelle Chieco and I am a 29 (almost 30!) year old with Beta thalassemia major. I grew up in Westchester NY with my amazing parents and two sisters. I was diagnosed at 8 months of age and received my first blood transfusion right before my first birthday. My parents faced challenges in trying to find a hematologist who was knowledgeable of the condition. We were lucky to find a local hematologist who was skilled and also to have the guidance of Dr. Cohen at Children's Hospital of Philadelphia. I always received treatment at a small community hospital which often meant very long transfusions and no exposure to other children with thalassemia.



Michelle as a toddler with parents Peter & Rose Ann Chieco

Early adulthood was difficult, I definitely struggled with compliance to my desferal pump. I was lucky that the oral chelators were just starting to come to market and I was able to receive one prior to FDA approval with the help of the Foundation. My



Michelle and Alicia at Disneyland

"The Cooley's Anemia Foundations is an integral part of my life. The Foundation, along with my parents, was instrumental in how I looked at the disease. It was never a negative thing for me; it only led to new opportunities."

– Michelle Chieco

family was supportive during this time but also worried about if I was truly taking the best care of myself. I eventually realized that I had to be put my pump on or take my medicine for myself, not because my doctors and parents were telling me to do it. If I wanted great things out of life, it was up to me to make that happen by taking good care of myself.

I've carried that perspective with me for many years. Even on the days where I am too tired to wear my pump or don't want to take another pill I know I am making the right decision for myself. I am doing it so I can live a long full life, spend time with my husband and family and ultimately start a family of my own.

The Cooley's Anemia Foundations is an integral part of my life. The Foundation, along with my parents, was instrumental in how I looked at the disease. It was never a negative thing for me; it only led to new opportunities. From traveling to different patient conferences, to fancy dinner dances and getting to go on the Columbus day float, it always was a positive fun experience.

The most valuable part of the Foundation is the connection it provides to other patients through events like the CAF Pa-

tient-Family Conference. Meeting other patients my age when I was a school-age child really was incredibly powerful. I will never forget meeting Alicia Somma-Hodgskin at Disneyland many years ago. Her positivity about thalassemia made me feel like I could also have that same outlook. Meeting Ralph and learning that he was a nurse was a direct reason why I became a nurse as well.

I am passionate about being a nurse to patients with chronic conditions similar to thalassemia. I am passionate about ensuring they, too, know they can have full lives if they take care of themselves. I especially enjoy getting to know my patients over the years and seeing them grow up and helping them through the difficult young adulthood years.

Thanks to my amazing husband and incredible family, I am thriving. They give me positivity and a constant reminder that I need to take care of myself so I can be there for them for many years to come.

I think the most important thing I want the thalassemia community to remember is that thalassemia is not a death sentence. It is a treatable disease that can be managed. It will be difficult at times and there are sometimes complications but staying ahead of things and being your own best advocate is the best way to live a healthy life. People with thalassemia can truly do anything they want in life!



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 so that parents/patients can know about possible options they may wish to consider. CAF does not endorse one camp over another. We recommend visiting the camp websites for more detailed information about the camp sessions, planned activities, dates, amenities, and for application instructions.

THE PAINTED TURTLE – LAKE HUGHES, CA

July 18-22, 2019: Camp for Children with Thalassemia, Hemophilia and Von Willebrand Disease
www.thepaintedturtle.org

THE DOUBLE H RANCH – LAKE LUZERNE, NY

May 4-6, 2019: Hematology/Oncology Family Weekend
www.doublehranch.org

CAMP KOREY – MT. VERNON, WA

Summer 2019 schedule coming soon
www.campkorey.org

HOLE IN THE WALL GANG CAMP – ASHFORD, CT

June-August, 2019: Summer Camps for General Diagnoses
www.holeinthewallgang.org

ROUNDUP RIVER RANCH – GYPSUM, CO

July 8-13: Camp for Children with Blood Disorders and Other Diagnoses
www.roundupriverranch.org

VICTORY JUNCTION – RANDLEMAN, NC

June 23-27, 2019: Children with Genetic and Neurological Disorders
www.victoryjunction.org

NORTH STAR REACH – PINCKNEY, MI

July 31-August 5, 2019: Sickle Cell, Hematology, and Pain Management Camp
www.northstarreach.org

CAMP HOLIDAY TRAILS – CHARLOTTESVILLE, VA

June-July, 2019 – Family Camp Weekends
www.campholidaytrails.org

CAMP BOGGY CREEK - EUSTIS, FL

June-July, 2019 – Summer Camps
www.boggycreek.org

FLYING HORSE FARMS - MT. GILEAD, OH

July 27-August 1- Hematology/Oncology/Rare Disease Camp
www.flyinghorsefarms.org

COMMUNITY CELEBRATES CAF'S THIRD ANNUAL THRIVING WITH THAL WEEK

This past June, CAF held our third annual Thriving with Thal week in celebration of the tenacity of thalassemia patients around the world. While living with thalassemia comes with many challenges, patients find ways to thrive every day. This year, we created a special #ThrivingWithThal frame on Facebook so that participants could frame their photos and add a caption on how they are thriving with thalassemia. Check out more of these photos and accompanying stories by searching under the hashtag #ThrivingWithThal on Facebook, Instagram and Twitter. We thank all who participated for sharing your inspiring stories!



"I am 53 years old and soon to be a Grandma!! A day that we thought would never be possible because of my thalassemia. But 8 years ago I finally admitted that I needed help. I couldn't do it anymore with a hgb in the 8's and, therefore, I became transfusion dependent. With the continual support of my husband and family, I am stronger now to face each day, its trials and the challenges of thal. I refuse to let it stop me. Some days, it will slow me down, but two bottles of red pick me back up again. I will thrive and survive, 'cause I have a granddaughter who needs to be loved and spoiled by me."
- Gracemarie



"My name is Emanuel and I will be turning 51 at the end of August. I was born in the U.S. and am from Greek descent. Thanks to blood donors, every month I receive three to four units of red cells. To manage my iron levels, I chelate with ExJade every day. I have a wonderful 12-year-old daughter and I am proud to be thriving with thalassemia." - Emanuel



"Madison and MaeLeigh are the epitome of strength, resilience and joy. They enjoying cheerleading, riding bikes, dance parties in the kitchen, s'mores over the campfire and hanging with their big brothers. They have the ability to make the darkest day brighter. While at times they become discouraged thinking of a lifetime of transfusions, pokes, medications, and



"Grow through what you go through, there's always a reason to smile."
- Priyanka

"My daughter actually chose to get her transfusion on her 18th birthday because the nurses at Children's Hospital have become like family. Now she's graduated high school and ready to go to college to be a chef. We all have different struggles in life, learning to see life's beauty and joys is the key."
- Ingrid (mother)

MRI's, they understand they are more fortunate than many. Our family is extremely grateful for Cooley's Anemia Foundation. Not only their research and support, but also for the annual Patient and Family Conference. We live in a very rural area and to my knowledge there aren't any other individuals with thalassemia near us. So the CAF Patient-Family Conference is the only time Madison and MaeLeigh have the opportunity to interact, play with, and learn from others with thalassemia."
- Jill Marie (mother)



"My name is Nusruth, and I live in Vacoas-Phoenix, Mauritius, where I was born and raised. I don't remember when I was diagnosed with thalassemia major, but my parents tell me I was a baby of 4 months. I've been under treatment for as long as I can remember. Part of that treatment requires regular blood transfusions. Another part of the treatment involves medicines that get rid of excess iron in my body, which come from all those transfusions."

From infancy to age 20 years, I have taken medicine called deferoxamine. The only way to take this particular medicine is to inject it into your body over a long period of time which is painful and boring. Now instead of injections I take a new oral medicine called Asunra (deferaxirox) that I take every morning which makes my schedule a lot more flexible.

I am thankful that treatment is becoming more manageable for people with thalassemia. A pill is definitely much easier to take than a 12-hour shot every night. Maintaining an appropriate treatment routine is my priority. It can be really challenging at times, but my health is worth it.

Blood donations are literally a lifeline for those who need regular transfusions like people with thalassemia, leukemia and other conditions. For me, blood is a unique gift of life. It gives me the opportunity to live. It gives me the opportunity to go to work. It gives me the opportunity to be happy. It gives me the opportunity to study, to travel and to get a job, to watch a bit of telly, have a milkshake and so on... Every single bag of blood is priceless. I never know whose blood I received, so as far as I'm concerned, every blood donor is saving my life. I thank God for each of you." - Nusruth

"My name is Zalkifel, and I was diagnosed with thalassemia major at 6 months old. Since then, I have received blood transfusions approximately every 21 days. It was never easy, and even my relatives and some doctors said that I wouldn't survive long. But my mom had a different view of me in her mind, and she nourished me in every field of life. I was a swimmer throughout school and went to a prestigious college, the University of Karachi in Pakistan.

I had a tough time throughout my journey, but I never opened up to anyone about my condition. I had to chelate for around twelve hours daily through an intravenous injection, attend classes, travel in buses on long routes and much more with hemoglobin levels sometimes as low as 6 and 7. The reason I never talked about my condition openly is because I never liked sympathy from anyone. I believe I can challenge myself and I can push my limits, and that's the beauty of our willpower. You never know how far you can sail on this boat called life. I never demanded any favor, or an escape route, or even flexibility because of my thalassemia. I want to set an example that you don't need cheat codes to achieve anything in this game of life. A little courage and consistency can take you to the stars.

I lost my brother and my best friend in my second year of Pharmacy because of thalassemia, yet it gave me more courage. Apart from being a professional in my field, I am going train myself as a speaker and a coach. InshaAllah! Always remember, we all get tested in different ways and intensities but a beautiful portrait is never made just after few strokes. It takes a lot. Silence gives you calmness, troubles give you strength, hardships give you maturity and experience gives you wisdom. You are not known by your problems but the way you face them. If you think that only you have problems, think again! Know that if you have the will to stand, friends to cheer you up, a family to support you and a vision to achieve a goal, then, my friend, the game is still on for you!" - Zalkifel



CAF CELEBRATES NATIONAL ADOPTION MONTH

Last year in November, CAF began a tradition of celebrating National Adoption month by sharing information about adoption with the community. This November, we continued that tradition with the goals of bringing attention to the need for permanent families for children with thalassemia, providing the greater community with resources and information on thalassemia adoption, and sharing the stories and perspectives of adoptive families.

Right now, there are some very special children with beta #thalassemia who are waiting to be adopted by a loving family. CAF is happy to share information about them along with resources for families who are interested in adoption.

Duncan was born in China with beta thalassemia major and is transfusion dependent. He needs a blood transfusion approximately every 3 weeks and receives iron chelation daily. Unfortunately, Duncan has not been able to attend school outside of the orphanage due to his needs, but does receive schooling inside the orphanage. Duncan is very polite and gets along well with other children. A forever family and the medical care and education Duncan could receive in the United States would be life-changing for this amazing little boy.

Bradley is a kind and friendly 10-year-old boy in China with beta thalassemia major. He is being transfused every 1-2 months and is receiving daily chelation treatment to manage his iron levels.

Grace is a sweet 10-year-old girl in China with beta thalassemia major and Dandy Walker syndrome. She is receiving blood transfusions and is on chelation medication to manage her iron levels. Grace currently lives with a foster family and is waiting for her forever family.

To learn more about these and other waiting children with thalassemia, email: brooke@madisonadoption.org. Madison Adoption Agency has support grants of up to \$4,000 for any family who says yes to a waiting child.

CAF also has a \$1,000 support grant for adoptive families. Visit thalassemia.org/adoption for information about the CAF Adoption Support Grant, stories of adoption, answers to frequently asked questions about thalassemia adoption, and for other helpful resources on adopting a child with thalassemia.



Duncan



Bradley



Grace

BLUEBIRD BIO AND TRACY ANTONELLI HONORED AT CAF ANNUAL GALA

On Thursday, June 7th, Cooley's Anemia Foundation hosted our annual Gala at the Lighthouse at Chelsea Piers in NYC. Nearly 300 guests attended, helping to raise over \$385,000.

Nick Leschly, chief bluebird of bluebird bio, accepted the Humanitarian of the Year Award on behalf of the company, which is conducting clinical trials in gene therapy for beta-thalassemia. Upon presenting this honor, CAF National President Peter Chieco remarked, "For years, we have championed gene therapy as a potential curative approach to thalassemia. We are thrilled to recognize the exceptional work and dedication of bluebird bio in this exciting area. bluebird bio's commitment gives hope to thalassemia patients here in the United States and around the world."

"We could not have made the progress we've made, or look to the road ahead, without the tremendous partnership of the thalassemia patients and their families who have participated in our clinical studies, the study investigators, and patient advocacy organizations who come together with a common purpose to advance treatment."

– Nick Leschly, chief bluebird, bluebird bio

"bluebird is deeply honored to receive this year's Cooley's Anemia Foundation Humanitarian Award and we accept it on behalf of all living with thalassemia," said Nick Leschly, chief bluebird, bluebird bio. "Our work is just beginning and we are dedicated to proving our belief that a one-time gene therapy holds the potential to transform the treatment of thalassemia and improve the lives of patients. We could not have made the progress we've made, or look to the road ahead, without the tremendous partnership of the thalassemia patients and their families who have participated in our clinical studies, the study investigators, and patient advocacy organizations who come together with a common purpose to advance treatment."

CAF also honored Tracy Antonelli with the Patient Recognition Award in acknowledgment of her triumphs over the challenges of living with thalassemia and her adoption of three children with thalassemia from China. Tracy is part of a new generation of thalassemia patients who, thanks to advances in care, are living longer, fuller lives. In years past, patients had little hope of living far into adulthood, getting married or starting careers. With newfound hope, people like Tracy are overcoming their obstacles

and enjoying life experiences previously closed to them. One way in which Tracy has chosen to share this sense of hope is through adoption of children with thalassemia who would otherwise have little chance of growing and thriving.

Of Ms. Antonelli's recognition, Mr. Chieco said, "Many children in other parts of the world do not have access to the exceptional standard of care that is available here in the United States," said Mr. Chieco, adding that about 10% of patients in the Cooley's Anemia Foundation database are adopted from overseas. "Tracy's selfless sharing of herself as a mother who can personally relate to the struggles these children face is an inspiration. These adorable children are blessed to be brought up by someone who is in all ways an exemplary role model; their lives and futures have been altered in an amazing way."

"I am proud of the achievements I have made in my life professionally in the field of clinical research, but my proudest accomplishment is being a mother to these amazing children," says Tracy Antonelli. "I knew from when I was very young that I wanted to adopt a child from China. When I learned that children were being abandoned because of their thalassemia, my mind was made up. I am so honored and humbled to be recognized by the Cooley's Anemia Foundation. Thanks to years of tireless work by the Cooley's Anemia Foundation, I know that the future is very bright for my daughters."



Back row: Frank Fusaro, Dinner Chairman and Emcee; Patrick Mooty, husband of Patient Recognition honoree; Tracy Antonelli, Patient Recognition honoree and Member Cooley's Anemia Foundation Board of Directors; Nick Leschly, Humanitarian of the Year honoree; Peter Chieco, National President of CAF Board of Directors. Front row: Rosie, Emmie, and Frannie, children of Tracy Antonelli and Patrick Mooty



Gala attendees looking at silent auction items during the cocktail hour



Nick Leschly, chief bluebird of bluebird bio, accepting the Humanitarian of the Year Award



Left: Lisa Pouchie, Gala Committee Member, and Michael Pouchie, Member of the CAF Board of Directors and Gala Committee Member
Right: Peter Chieco presenting Nick Leschly with the award



Rose Ann Chieco, Deanna Chieco, Michelle Chieco Lenz; Member, CAF Board of Directors, Jennie Chieco, and Peter Chieco, National President, CAF Board of Directors



Julia Cenzoprano, Member, CAF Board of Directors; Janice Cenzoprano, National Vice President, CAF Board of Directors; Anthony Cenzoprano; and Matthew Cenzoprano

CARE WALK 2019 GEARS UP FOR A BIG YEAR AHEAD

This past May, thousands of caring advocates gathered in cities across the U.S. to Care Walk in honor and support of all those living with the challenges of thalassemia, raising over \$310,000 for the Foundation.

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, 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.

As we start the process of launching Care Walk 2019, we would like to take this opportunity to thank all of our volunteers for the incredible work they have done to grow Care Walk and make it the success it has become - and the even bigger success it is going to be this year.

EARLY REGISTRATION NOW OPEN

The official Care Walk launch date is January 11, 2019. However, we are making early registration available to give Captains more time to set up team pages.

Captains who register their teams by December 31 will receive a special Care Walk captain baseball cap!

Visit bit.ly/carewalk2019 to register your team today.

JOIN OUR FACEBOOK GROUP FOR CAPTAINS

Exchange Care Walk ideas and advice, and get to know your fellow Captains by joining our new Facebook group for Care Walk Captains. May will be here before we know it, so let's all come together and share planning strategies in the months leading to the big day!

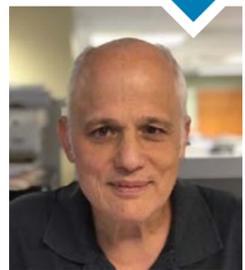
Join at bit.ly/CaptainsGroupFB.

A LOOK BACK ON CARE WALK 2018



DOUBLING UP OR "BUNCHING" YOUR CHARITABLE CONTRIBUTIONS

The new Tax Cuts and Job Act is upon us, effective for the year 2018, and will bring many changes in the tax code. This new tax bill will shrink the number of households claiming itemized deductions on their tax returns due to limiting and eliminating many prior allowable tax deductions. The standard deduction will also rise to \$12,000 for single tax filers and \$24,000 for married couples in 2018. Both of these changes will lead to more strategizing of charitable contribution giving, especially if there is a loss of a tax savings.



Dean Hernan

One strategy to combat the new tax law is to consider doubling up or bunching your charitable gift giving to every other year or two out of three years. Doubling up or bunching your gift giving may allow you to itemize your deductions in the years you give, instead of taking the standard deduction for that year.

Lets take a look at this example. Consider a married couple claiming the maximum property and state income tax deduction of \$10,000 along with mortgage interest of \$10,000 per year. They will need to have \$4,001 of charitable gifts per year in order to surpass the \$24,000 standard deduction threshold. (Taxpayers can deduct the higher of their actual itemized deductions or their standard deduction). If this couple normally gives \$3,500 to charities annually, doubling up the contributions one year (to \$7,000) and skipping any contribution the following year will have the following result: In the year of doubling up, the couple will have itemized deductions of \$27,000 (\$10,000 in state and local taxes, \$10,000 mortgage interest and \$7,000 in charitable contributions) instead of a \$24,000 standard deduction for that year and the following year will have the same \$24,000 standard deduction they would have had they made their donations at their normal \$3,500 per year. The doubling up resulted in reducing their taxable income by \$3,000 in the year of doubling up while having the same taxable income in year two. Had they made their normal \$3,500 per year in donations, their total itemized deductions for both years would have been \$23,500, which is less than the annual standard deduction allowed.

Not-for-profits will be facing many uncertainties under the new tax law. Doubling up or bunching your annual gift giving may be mutually beneficial for the donors as well as the not-for-profits (such as the Cooley's Anemia Foundation).

Please consult with your personal tax advisors before implementing this strategy. If you want to consider doubling up this year, please make sure the additional donations are made by December 31, 2018

Have You Chelated Today?



Iron overload from chronic transfusions is a major cause of complications and shortened lifespan in thalassemia.

Staying on track with chelation therapy is the best way to prevent iron overload and stay healthy. Please discuss all available chelation options with your doctor. The best iron chelation treatment plan is one that you are able to stick to!

This message was developed as part of Cooperative Agreement #5N027DD001150-04-00 from the Centers for Disease Control and Prevention.

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

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:
Henry Ackermann, National Development Director,
Cooley's Anemia Foundation, 330 Seventh Avenue, Suite 200, New York, NY 10001

Telephone: (212) 279-8090, ext.204
 Email: hackermann@thalassemia.org

END-OF-YEAR CONSIDERATIONS

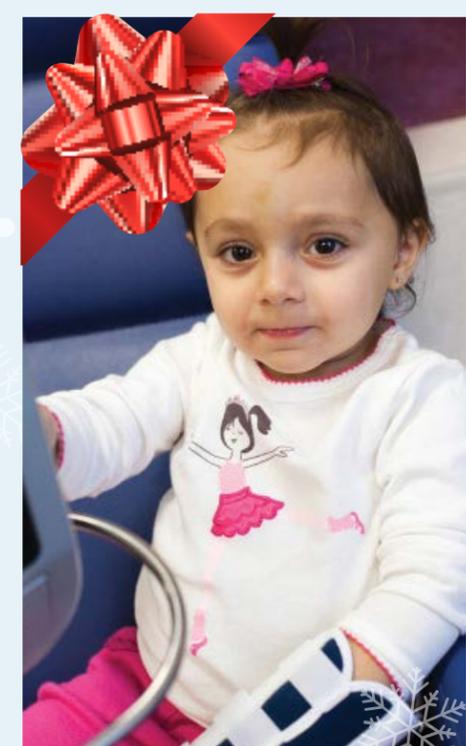
By Henry Ackermann, National Development Director

Before you know it, 2018 will be coming to an end! And 2018 is the first year that the full impact of new tax laws take effect. So here are some things to consider as you ponder your year-end giving to Cooley's Anemia Foundation:

- **IRA Rollover –**
 For those who will turn or have turned 70 ½ years old in 2018, welcome to the IRA required Minimum Distribution Club! You now have to annually withdraw a fixed amount. And you have to pay income tax on the amount withdrawn – unless you have your fund manager give the entire distribution directly to a charity (like CAF). This is called a Charitable Rollover and the tax on this amount is waived! Talk about a win/win – the charity gets the contribution and you avoid being taxed. Contact your IRA fund manager for details.
- **Stock Gifts –**
 For some, 2018 has been another good year! And you and your favorite charity (Cooley's Anemia Foundation) can benefit from donating shares of appreciated stock to the charity. Another win/win – CAF sells the shares of stock and gets the proceeds and you do not have to pay capital gains on the appreciated stock. Contact your broker for details.

- **Annual Appeal –**
 For all of us, no matter our age or tax position in 2018, we have an excellent opportunity to contribute to the good work of Cooley's Anemia Foundation by donating during the Annual Appeal. From Thanksgiving through the end of the year, we have a chance to be magnanimous to our loved-ones, colleagues, and friends. We can also be generous to those served by our favorite charity, so remember CAF in your end-of-year giving!
- **Legacy Society –**
 New for 2018, Cooley's Anemia Foundation has inaugurated a Legacy Society. Members of the Legacy Society are those individuals who let us know that they have arranged for CAF to receive a gift as part of their final plans. There are many ways to arrange for such planned gifts and they are outlined on the sign-up form on the next page. Signing-up to join the Legacy Society is merely an indication of your intent to remember CAF with a planned gift, not a legal obligation to do so. So please consider joining. Your name(s) will be added to a growing list of planned givers. We will have much more information to share with you soon about the benefits of joining the Cooley's Anemia Foundation Legacy Society. Thank you!

WITH YOUR HELP, THE CURE IS WITHIN REACH!



THIS HOLIDAY SEASON, 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.

JOIN THE "PUSH-UPS TO FIGHT THALASSEMIA" CHALLENGE!

Kick off the new year by challenging yourself, your coworkers, family members and friends to a set of push-ups to raise thalassemia awareness and funds needed to help our patients. Complete the challenge between January 1 and February 9, and be sure to share your pictures on social media using

#PushUpsThal.

**REGISTER TODAY AT
BIT.LY/PUSHUPSTHAL**



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