



SPRING 2018

LIFELINE

COOLEY'S ANEMIA FOUNDATION • LEADING THE FIGHT AGAINST THALASSEMIA

CARE WALK 2018 BRINGS THOUSANDS TOGETHER IN CITIES ACROSS THE U.S.

This May, thousands of supporters across the country will come together in cities across the U.S. to Care Walk in honor and support of all those living with the challenges of thalassemia. Care Walk is the Cooley's Anemia Foundation's largest annual fundraising event and opportunity to bring together the thalassemia community and its supporters around the country. Funds raised support thalassemia medical research, patient support services, and education initiatives to raise awareness of the genetic blood disorder.

Care Walk is a unique fundraising event for the Foundation because rather than taking place in one location, this event

brings people together in their own communities. We have some of the most incredible volunteers who take the initiative to plan these walks and to invite their friends and neighbors to join the fight against thalassemia on a local level. These volunteers select the location and organize details like where the group will walk and who will bring snacks. Some of these walks take place around a lake or park and involve hundreds of people, and others take place in more intimate locations like neighborhoods with individual families. Besides walking, the activities featured at these Care Walks range from live musical performances, bounce houses, and face painting to silent auctions, barbecues and blood drives. The one thing they have in common is lots of fun!

All of the work our volunteers put into planning their Care Walks, soliciting donations in person and online, and educating the community about thalassemia has made Care Walk have the most impact of any single event of the year for the Foundation. We want to thank each of our volunteers, participants, donors and sponsors for your dedication, support and friendship. To join Care Walk 2018, visit www.bit.ly/carewalk2018.

Continue on page 14 



Last year's Care Walk in Orlando, FL hosted by Charishma and Harry Chulani.

CAF EXECUTIVE COMMITTEE MEMBERS

Anthony J. Viola
President

Amy Celento
Vice President

Janet Kwiatkowski, MD, MSCE
Medical Advisory Board Chair

Dean Hernan
Treasurer

Janice Cenzoprano
Secretary

Cammie Brandofino

Mary Ann Cervoni-Iaia

Ralph Colasanti

Terri DiFilippo

Robert Ficarra

Maria Hadjidemetriou

EXECUTIVE DIRECTOR

Craig Butler

LIFELINE EDITOR

Mary Woldegiorgis
National Communications Director

LETTER FROM TONY VIOLA, COOLEY'S ANEMIA FOUNDATION NATIONAL PRESIDENT



Dear Friends,

PHEW!! Where did these 10 years go? I started my National Presidency tenure in May, 2008 and, with sadness in my heart, this part of my CAF journey will end with the next Board of Directors meeting in May, 2018, when I pass on leadership of the Foundation to Peter Chieco, who has been nominated to the office of National President (and who will do a tremendous job).

So where do I begin? How about we start with the very beginning? It was January, 1971, the month that my cousin Nunzio Cazzetta passed away from complications of Cooley's anemia. At the time, I was 11 years old, and Nunzio was a month younger. I knew that Nunzio was "different," but I did not know why, until his passing. At that point, it was discussed as to what had taken place and how it eventually happened. That was my first indoctrination into what would become an adult lifetime of volunteer service for a great organization.

After Nunzio's passing, I got closer to his younger brother, Ralph Cazzetta, mostly because I started to understand what he might be going through as a Cooley's patient. We, as non-patients, can never fully understand the day-to-day trials and tribulations that occur, but at least the offer of compassion can be displayed and relayed to the receiver of such unknown daily routines. Between my two cousins and my aunt and uncle, Rose and Nunzio, with whom I have been very close over my lifetime, I started to become interested in CAF.

Getting to know the patients, their families, and the Cooley's Anemia Foundation, I officially started my volunteerism in a small way by doing what I do best, offering my accounting services to help out and minimize accounting costs to CAF, in 1989. That eventually led to helping out with small tasks and eventually joining the National Board during the 90's. I would be remiss if I did not single out Gina Cioffi, the great National Executive Director for 20 years who mentored me on my path of becoming a CAF volunteer and eventually National Officer.

It was in 1996 that I commenced on my "serious" journey with CAF. In August, 1996, Thomas Nastasi, Dean Hernan, and I held a golf outing that raised \$40,000 for the Foundation, with the invaluable assistance of our greatest fundraiser, Tony Laurino. That was my first fundraising endeavor. We held the 2nd golf outing the following year and we did decently but that event just did not seem to me to be a long-term project.

So Dean and I discussed what annual event we could come up with that would raise significant funds for CAF. We then involved Tony Laurino and Frank Fusaro, which led to our Annual Cigar Night, a fairly unique event that attracted very generous donors and friends. The first Cigar Night was held at the Columbus Club in 1999, with approximately 100 people attending. Seeking to expand the number of people who could attend, we moved our event to the Grand Havana Club the following year. From there the event took off. During the Cigar Night's existence, from 1999 to 2013, we raised approximately \$2.5M for

the benefit of our patients. While Dean, Frank F., Tony L., and I started this event, it was generous people such as Frank Marzano and Ron Purpora that helped take this to another fundraising level. All involved with this event should be very proud of their accomplishments.

During the years of the Cigar Night, I eventually was placed into the role of National Vice President by then National President, Frank Somma, my predecessor. It was during my terms as National Vice President that I strongly considered doing even more for CAF.

Fast forward to May, 2004. My cousin, Ralph Cazzetta, passes away a day after his 39th birthday. Ralph had 28 years more than Nunzio did to live his life. Ralph also had something that Nunzio did not have, and that was a daily treatment option to help alleviate the iron build-up that the endless blood transfusions caused. That treatment option was Desferal, distributed by Novartis, a company that has helped extend many thalassemic lives around the world. This allowed Ralph to accomplish his life-long goal of becoming a Registered Nurse, for which Ralph's family is eternally grateful. And while we, as a family, were "relieved" that Ralph attained his goal, we were also angry as well. It was this exact day, May 19, 2004, that I decided to run for the National Presidency after Mr. Somma was ready to step down.

After discussions with Frank Somma and some of the Officers of CAF, I decided to ask for nomination for the role of National President in May, 2008. Being named National President of the Cooley's Anemia Foundation was one of the proudest moments of any part of my life. I was so motivated to do my very best for all involved.

Right from the beginning, my agenda was centered around the concept of "patient care" and "patient quality of life." My first agenda was greatly influenced by Gina Cioffi who helped me mold my thoughts and opinions into a decipherable context that made sense. Of course my uncle, Nunzio Cazzetta, also had a big say in the direction that I would head into, to try and make a difference while doing so effectively and efficiently.

My first order of business was to right a tremendous wrong, here in the United States. Desferal was joined by Exjade, an oral chelator, as treatment options, but our patients still needed more ways of getting the iron out of their systems, as no one daily chelator works for every thalassemia patient. So, along with Gina Cioffi, I sought to get the drug, Ferriprox (formerly called L1), passed through the FDA. This part of my CAF life started in the fall of 2008.

Without going into the details, after many days of heartache, stress, and sometimes absolute torture, the FDA finally held a meeting to consider Ferriprox in October, 2011. When the vote came up on the screen in the Washington, DC hearing, 10-2 in favor of recommending passage, the tears that were shed were for joy and utter relief, that this nearly 15 year crusade of the Foundation had finally come to an end. There were so many people involved in this success, but the late Barry Sherman (may he rest in peace), Michael Spino, and Fernando Tricta, all from ApoPharma (the company that manufactures Ferriprox), deserve credit for persevering where lesser men would have given up. Many, many thanks to them for their heroic efforts. On a personal note, I have always believed that had Ferriprox been available years earlier, my cousin Ralph's life would likely have been saved. This was another motivation, for getting Ferriprox passed through the FDA. For me personally, this was my greatest accomplishment outside of my family life, and that includes my business career.

Throughout my nearly 30 years of volunteer service, I have met so many people through CAF. CAF staff, patients, families, CAF contacts, doctors, researchers, pharma, donors (including organizational donors), government agency employees, etc. Too many to acknowledge them all. So many contribute to the success of CAF, in its drive to make the patients' lives better every single day. We have raised millions of dollars over the years that I have become a full-fledged fundraising volunteer for CAF. Here is a listing of some acknowledgements, and please, do not feel insulted if you do not get mentioned specifically, as you all have had an influence in my CAF journey:

- My start into CAF: My cousins, Nunzio and Ralph Cazzetta, and their parents, Nunzio and Rose Cazzetta. Gina Cioffi, National Executive Director for 20 years. I do not have to say what you mean to me because I say it all the time. But thank you and I love you.
- The people responsible for my fundraising successes: Dean Hernan, Frank Fusaro, Frank Marzano, Tony Laurino, and all my family, friends, and business contacts who have generously given year after year to CAF. The four individuals named in this section shaped my life. They are about as important to me as any individuals can be. I would not be who I am today without them, for good or for bad, lol. I love you all.
- The CAF staff: To the best staff any nonprofit organization could ever have. First led by Gina Cioffi at the start of my volunteering career, and now led by Craig Butler, with whom I enjoyed working, even for a short time. Craig is exactly the right person to succeed Gina, one of the true stars of CAF. Tony Laurino, who had no direct connection to CAF, other than through his life-long friend, my uncle, Nunzio Cazzetta, has gone on to an over 50 year successful fundraising era. And the rest of the CAF staff, Eileen, Sandy, Kathleen, Zofia, Henry, Mary, Darlene, and Jonathan, with whom I enjoyed working over the years. My love to all of you. The respect that I have for all of you is indescribable.
- CAF Board and Executive Committee: I got to sit with the true heroes of CAF. Bob Ficarra, whose father Frank Ficarra started CAF back in 1954, has over 50 years of CAF service

and dedication, unmatched by anyone else currently in CAF. Amy Celento, my National Vice President during my 10 year term, I thank you for all your support, assistance, and love for me and my family. Thank you to Janice Cenzaprano, my National Secretary and daughter to CAF royalty, the Paradiso Family, for doing the job that no one else would do. Dean Hernan, my best friend and National Treasurer, who has been there for me my whole adult life. Cammie, Terri, Mary Ann, Maria, Ralph, etc., thank you all for everything that you have done. And thank you to the National Board, many Directors who are friends of mine that have stepped up to the plate in numerous ways to get CAF where it is today. The two Mikes, Tom W., etc., thank you. Love to all in this category of appreciation.

- CAF contacts and partners: Lyle Dennis, our legislative advocate, thank you for 20 years of tremendous service. CAF does not get the meetings we get with NIH without you. Our physicians and researchers that I have come to know, Elliott, Ellis, Alan, Janet, Tom, John, Vasili (remember when you rolled your eyes at me at ASH 2007 when I told you that I was the next CAF President?), Grady (I miss you Bob...), Dr. G, Michael, all the international physicians, etc. Thank you all, I look forward to seeing you soon. Michael and Fernando, what can I say? I tell you every year. Love you all.
- To the donors: Wow, where to begin? The Order Sons and Daughters of Italy in America were there from the very beginning. Over 40 years and over \$2.5M of donor contributions to CAF and the patients they serve. Again, words cannot describe how I feel about this great organization and the great people who participate in it. I have gotten to know a lot of you and I have become one of you. Thank you for all you do and the friendship that you have extended to me and my family. UNICO, another organization that has stepped up for many, many years. And now we have UFOLI (the 7-11 stores) and the Milana Family Foundation (my friend Thomas and Adrianna Milana, along with their 5 beautiful daughters). My friends, my family, and my business contacts, I cannot thank you enough, from the bottom of my heart. I have always honored your generosity by using those dollars in the best way that I knew how. Thank you all and I love you.
- The parents of the patients: There are many that have influenced me over time, but among the parents that stand out are Peter and Rose Ann Chieco, Frank and Deb Somma, Cammie Brandofino, Terri DiFilippo, Mike and Sarah Connolly, etc. A lot of what I tried to do, was dedicated to all of you in this category of appreciation. Thank you for your support over the years and all my love to you and your families.
- Patients: I cannot name you all, trust me on that, but over the long-term, patients like Mary Ann, Maria, Michelle, Alicia, Zayna (I miss you...), AJ, Jennine, Ralph Colasanti, James, the patients I have met at the Patient Conferences, etc., all have been embedded into my heart, for the rest of my life. I promise to carry on the work of CAF and I will see

LETTER FROM TONY VIOLA (CONTINUED)

you all at the next Patient Conference in Atlanta. Words cannot express the love that I have for you and your families. I did my best and I hope, in some small way, I impacted your life.

- Finally, last but certainly not least, I could not do anything without my family. My wife Susan, who puts up with my “schedule” for my business and my volunteer work, but supports me every step of the way. Over 30 years of friendship and love that has turned into a successful life, not dictated by finances, but dictated by the results, a great marriage and two very successful sons, in Anthony and James, who we are very proud of. I love you more every day and I cannot imagine my life without you.

Thank you and I love you from the bottom of my heart.

Again, please do not be insulted if I have not named you specifically, because there are many people who have influenced my adult life and my CAF volunteerism. For that I thank you. This is not an end to my CAF career, just a modification. My wife Susan says to me “so what’s going to change?” LOL. Enough said. Thank you and good luck to Peter Chieco, the best person I can think of to lead CAF for the foreseeable future.

With all my love, prayers, and dedication,
Tony

Pregnancy in Thalassemia

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



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

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

This message was developed as part of Cooperative Agreement #SNU27DD001150-04-00 from the Centers for Disease Control and Prevention.

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

REGISTRATION NOW OPEN FOR 2018 PATIENT-FAMILY CONFERENCE

Registration is now open for CAF's 2018 Patient-Family Conference! This year's Conference will be held from July 6-8 at the Westin Atlanta Airport Hotel in Atlanta, GA. The 2018 Patient-Family Conference is a unique opportunity for U.S. individuals with thalassemia and their family members (parents, siblings, spouses, etc.) to meet other members of the community and learn important information that can have a positive impact on their health and their lives.

OFFICIAL REGISTRATION WEBSITE:
WWW.DOCBW.COM/MEET/CAF

Please register no later than June 12.

We will also be holding special Thalassemia 101 introductory session on Friday, July 6 at 4:30 for families who are new to the community or who may feel that they need a refresher in some of the “basics” about thalassemia.

There is no registration fee for U.S. patients, family members and health care providers attending the Conference. CAF is also pleased to make the Conference meals (Friday lunch and reception; Saturday breakfast, lunch and dinner; Sunday brunch) available free of charge to U.S. patients, family members and health care providers. Non-U.S. patients and families may attend at a rate of \$300 per person; non-U.S. patients should contact Eileen Scott at CAF (Eileen.s@thalassemia.org).

U.S. patients/family members who would like to attend the Conference but find travel expenses beyond their current means should contact Patient Services Manager Eileen Scott at Eileen.s@thalassemia.org no later than May 1, 2018.

CAF has also arranged a special hotel room rate of \$127.42 per night (\$109.00 + 16.90% tax). This rate is valid for up to 4 people in the same room. Reservations must be made by Thursday, June 7. For details on hotel reservations, visit www.docbw.com/meet/caf/hotel.

To register for the Conference and for additional details, visit the official registration website at the link below.

www.docbw.com/meet/caf

CAF AWARDS 26 STUDENTS WITH PATIENT INCENTIVE AWARDS TOTALING \$31,750

CAF is thrilled to announce that 26 students with thalassemia were awarded with a total of \$31,750 in scholarship funding as recipients of the CAF 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. 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.

“These students inspire me so much and they prove to the world that thalassemia will not stop them from living the great lives they are destined to live.” – Anthony Viola, CAF President

“Much of the work we do at the Foundation is focused on furthering medical research for better treatment options and for a cure,” says CAF President Anthony Viola. “However, we also work hard to improve the everyday lives of people with thalassemia in other ways like encouraging them to fulfill their greatest dreams. We began the Patient Incentive Awards program to support thalassemia patients pursuing higher education. These students inspire me so much and they prove to the world that thalassemia will not stop them from living the great lives they are destined to live.”

Yasmeen Anis is a fourth year undergraduate student at Flagler College, and is one of the 26 recipients of a Patient Incentive Award this year. “I have always dreamed about being an elementary school teacher, but I never knew if I would be able to do it with my thalassemia always making me miss school and classes to go to doctor appointments,” explains Yasmin. “I have had some incredible teachers and people in my life who have inspired and encouraged me to keep going and NEGU (Never Ever Give Up). The teachers that I have had have truly shaped who I am and I hope that one day I can do the same for my students. The CAF Patient Incentive Award is not only helping me achieve my dream of becoming an elementary school teacher, but is also helping all of my future students as well.”

CAF congratulates each of these remarkable students, and we wish them the very best as they pursue their careers.

Testing for Low Bone Mass Helps Prevent Fractures.

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



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

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

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

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

COOLEY'S ANEMIA FOUNDATION

CARE WALK
MAY 6, 2018

WWW.BIT.LY/CAREWALK2018



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.

ROBERT MANNINO WINS FIRST PLACE PRIZE OF \$100,000 FOR SMARTPHONE APP FOR SELF-MANAGEMENT OF ANEMIA



CAF congratulates Robert Mannino, PhD student at Georgia Tech and Emory University, on winning first place in the competition for the 2017 Student Technology Prize for Primary Healthcare. This competition showcases student innovations in primary-care technology with the objective of providing opportunities for promising engineering students to develop their careers while also improving primary-care delivery.

Robert, who was diagnosed with beta thalassemia major at 6 months old, has received frequent blood transfusions throughout his entire life. By the age of 16, he knew that he wanted to focus his career on finding ways to improve the lives of people born with thalassemia. Robert began his pursuit of this career by enrolling in the biomedical engineering undergraduate program at the Georgia Institute of Technology, where his academic excellence resulted in his selection as the recipient of the CAF-ApoPharma Distinguished Scholar Award. After receiving this honor in 2014, Robert explained, “Being in a hospital all the time growing up, and getting to know a lot of people with blood-related illnesses, I want to use my talents to help others. I have seen firsthand what medical innovation from diagnostics to therapy can do to improve the life of a patient. My ultimate goal is to use my degree and the skills I will attain in graduate school to become a biomedical engineer translating research into new diagnostics and therapies for blood diseases in an industry setting. I hope to focus on developing new tools to study hemoglobin diseases and to improve patient outcomes. It is an honor that the Foundation has chosen to help me to pursue my goal.”

Since then, Robert has soared in the field of biomedical engineering. He currently works in the lab of Wilbur Lam, M.D., Ph.D., associate professor of biomedical engineering and researcher in the Petit Institute for Bioengineering and Bioscience. Dr. Lam is researching improved ways to detect iron overload caused by frequent blood transfusions. As a

researcher in Dr. Lam’s lab, Robert has been designing devices that can detect iron overload indicators. This research could have a phenomenal impact on early detection of iron overload, particularly in developing countries where children who receive frequent blood transfusions have an increased risk of organ failure resulting from iron overload.

Robert’s prize-winning project, entitled “Noninvasive Inexpensive Smartphone App for Patient Self-Management of Anemia,” uses smartphone photos of a patient’s fingernails for diagnosing anemia. The initial clinical assessment of the app is now complete, and the \$100,000 prize money from this contest will support further development of Robert’s research, which is based on his PhD dissertation. In a recent interview with Children’s Healthcare of Atlanta, Robert remarked, “being able to detect iron overload early is an exciting opportunity... I don’t know anyone else who’s doing this kind of research.”

CAF congratulates Robert on this exciting achievement, and we are so proud of all his work to improve the lives of individuals with thalassemia.

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 #5NU27DD001150-04-00 from the Centers for Disease Control and Prevention.

AARON CHENG SELECTED AS CAF-APOPHARMA DISTINGUISHED SCHOLAR AWARD RECIPIENT

The Cooley's Anemia Foundation is proud to announce Aaron Cheng as the 2017 CAF-ApoPharma Distinguished Scholar Award recipient. This scholarship is in the amount of \$20,000 and is presented to individuals in the United States with a clinically significant form of thalassemia who are pursuing postgraduate doctoral level studies in medicine or science. This award is made possible through a grant from pharmaceutical manufacturer ApoPharma to encourage and support the pursuit of higher education by members of the thalassemia patient community.

"ApoPharma proudly supports Aaron Cheng, who, as a thalassemia patient, in pursuit of an advanced degrees in the Biomedical Sciences, is making a difference," says Dr. Michael Spino, President of ApoPharma, Inc. "Having seen Aaron's work presented at the annual meeting of the American Society of Hematology in 2016 demonstrates that he is a worthy recipient of the Distinguished Scholar Award. His further quest for training in the hematology patient community is notable. All of us at ApoPharma extend our best wishes and join in congratulating Aaron on this achievement."

Aaron received his undergraduate degree in Chemical and Physical Biology from Harvard College, and is currently a fourth year student at Harvard Medical School. Aaron's goal is to learn how to become a productive physician-scientist. In particular, he hopes to explore computational research because new advances in genetics and high throughput cell biology methods have made it imperative to gain a deep understanding of quantitative methods. Last year, he worked on 5 different patients with blood disorders that were not explained by common genetic mutations. To better understand the potential causes of their diseases, he learned how to use powerful computational tools to process many thousands of genetic variants that are present in the patients and their families, and applied genetic filters in order to identify several mutations that may explain their disease.

"Aaron is more than a promising young medical researcher; he is a leader in the thalassemia community with the type of proactive and positive thinking that has inspired so many to reach higher," remarks Anthony Viola, President of the Cooley's Anemia Foundation. "We are so proud to award him for the second time with the CAF-ApoPharma Distinguished Scholar Award. CAF thanks ApoPharma for their continued support of



"To me, this award serves as a reminder that although thalassemia is a challenge to deal with, it doesn't need to prevent patients from achieving their dreams." – Aaron Cheng

thalassemia patients, as well as the Scholarship Committee for their work reviewing the submissions."

Aaron's role as a caretaker of patients is also extremely important to him. Over the past year he has rotated through his clinical clerkships at the Massachusetts General Hospital, where he has been involved in the inpatient care of patients among several specialties. He hopes to supplement his clinical experiences in the years to come by exploring fields such as cardiology and critical care medicine to best serve patients in the future.

"I am honored to be chosen as the CAF ApoPharma scholar this year," says Aaron. "To me, this award serves as a reminder that although thalassemia is a challenge to deal with, it doesn't need to prevent patients from achieving their dreams. I appreciate the efforts that ApoPharma and CAF have dedicated toward encouraging higher education in patients with thalassemia, and I believe that this is a worthwhile and important investment. I will be putting the reward toward an extra year of hematology-related research at Boston Children's Hospital, so I may better develop my career as a physician and scientist."

CAF congratulates Aaron on his phenomenal achievements and we wish him much success as he completes medical school and continues his research.

VOLUNTEER SPOTLIGHT: CHARISHMA CHULANI

CAF launched our “Volunteer Spotlight” Series two years ago to highlight the stories of some of the many selfless volunteers who play a major role in making our work possible. We are truly grateful for all of the love and heart-felt effort each of our volunteers puts into organizing awareness events, blood drives and fundraisers, and we thank them for their commitment to improving the lives of individuals with thalassemia.

Charishma Chulani and her husband Harry Chulani are an inspiring couple who are both thalassemia carriers and parents to three children with thalassemia major, or Cooley’s anemia. They are hosting their second annual Care Walk this year in Orlando, FL on May 20 in support of individuals with thalassemia all over the country. CAF thanks Charishma and Harry for sharing their story with the community through this interview, and for raising thalassemia awareness through their Care Walk.

CAF: Could you tell us a bit about yourself and your family?

We live in Orlando, Florida, and I work as a part-time photographer. I’m 37 and I met my husband when I was 12 years old as our families were close friends. Our marriage was not arranged, though. We actually rarely ever spoke until I was 17 years old. We’ve been married for 14 years now and have three amazing children together, Aryan (Ari), Dylan, and Nia.

CAF: How did you learn that you are carriers of beta thalassemia?

My in-laws actually insisted I get trait tested because they already knew that my husband was a carrier. So I had no idea until I was 22 years old that I was a carrier. We did not really know much more than the internet told us at the time, which was very little and very negative. We were married two years later and just hopeful that the odds were in our favor.

CAF: What were the biggest questions, concerns and fears you had about raising three children with thalassemia?

I think we just worry about the unknown the most. Not knowing what the iron chelation medication may be doing internally is always in the back of my head since it’s all fairly new. Also, I fear that they could get bitter that the condition is life long, especially in the teenage and/or young adult years. Hospital visits are frequent and this will eventually interfere with their school, a job or social life.

CAF: What are the greatest joys of parenting three children with thalassemia?

The fact that all three have thalassemia major is disheartening, but at the same time it is really incredible as a mother to witness my children caring for each other the way they do when one of them has had a rough day. Also, as they grow they



“I’m so grateful to be able to spread awareness and raise funds for a cause that is this close to my heart.”
 – Charishma Chulani

won’t have the opportunity to compare themselves to the other siblings as they all share the same condition. I mean, no matter how you raise kids, it’s a natural thing for them to wonder why one has something that the other doesn’t. Each of my children has such a unique personality and inner strength. They are so different from one another! My hope is that they will collectively use these differences to get through the difficult stages of their life. It makes me happy knowing that they will have each other as support always.

CAF: What made you decide to begin your own Care Walk?

Well, out of the 1500+ people in the United States with thalassemia, I have three! I’m so grateful to be able to spread awareness and raise funds for a cause that is this close to my heart.

CAF: Why do you support the work of CAF?

We have received great support from CAF over the past 9 years I love being able to give back in some way and contribute in the effort toward researching how to make the lives of our loved ones better.

CAF: Do you have any advice to new parents of children with thalassemia?

I know from experience how difficult it is to digest the news that your child has any sort of health condition. Nothing truly alleviates this initial shock, regardless of how many children with thalassemia you have. Just know that thanks to research and modern medicine, thalassemia is manageable. Life is different but still beautiful and normal. Raise your children to see the beauty in the life they lead and see how fortunate they are always. Sometimes, we take it harder than our children and we just need to remember our kids are watching and learning from us! We have to keep a positive outlook and remind them that they are strong and capable of anything.

CAF: What is your greatest hope for your children and other children with this disorder?

Of course an easy cure would be great, but I honestly don't focus my thoughts on this. I just hope that they always see the positive and understand that in life, this is just a bump in the road. It doesn't define them and won't hold them back from their dreams as long as they stay positive and mentally strong.



VOLUNTEER SPOTLIGHT: BUFFALO CARES: THE BILA FAMILY BRINGS COMMUNITY TOGETHER FOR CARE WALK

Joycelyn Bila is hosting the third annual Care Walk in Buffalo, NY on June 3. Joycelyn began organizing this walk in honor of her daughter Josephine Bila and Kristina Wagar. This year, the Walk is dedicated to the memory of Michael J. LoCurto, who died this past December at age 46 due to complications related to thalassemia. We thank Joycelyn for sharing her story with the community, and for her dedication to educating the community about thalassemia through op-eds in newspapers, appearances on television news, and through the Buffalo Care Walk.

CAF: Could you tell us about yourself and your family?

I (Joycelyn) live in Buffalo with my husband of forty-three years, Nicolo. We have two beautiful, grown daughters; Josephine and Gloria. I am an artist and writer and my husband, a somewhat retired home improvement contractor. I met him through his friends who were dating my friends, while in high school.

CAF: How did you learn that you are carriers of beta thalassemia?

When I was seventeen I started becoming weak and jaundiced after months of treatment with iron and B12 injections. My general physician was very concerned since he couldn't figure out what was causing this. After being hospitalized, I was seen by a hematologist who performed a bone marrow test to check for thalassemia. The test results showed that I was a carrier of the thalassemia trait and therefore, had thalassemia minor. When Nick and I got married, we were told there was a chance we could have a child with thalassemia, so we went for genetic counseling. That was when we found out he carried the thalassemia trait as well. When I got pregnant there was concern that the baby would have thalassemia since there is a one in four chance that could happen with every pregnancy. We were devastated when Josephine was born with thalassemia major.

CAF: What were the biggest questions, concerns and fears you had about raising a child with thalassemia?

First, no one on either side of our families had ever heard of thalassemia, never mind anyone ever having it. It was very frightening to think that our child needed blood transfusions to survive along with learning about all the challenges she would face, physically and emotionally. We wondered what would life be like for her as a child with what was then and perhaps even now considered a disability? How would she go to school if she needed to have blood transfusions? Would she be able to keep up? How would other children treat her? What if she gets a fever or any kind of illness, what will be the outcome?



CAF: What challenges did you face?

I remember when Josephine started kindergarten she had an enlarged spleen. The teacher, knowing Josephine's condition and that she had thalassemia, wouldn't allow her to play on the playground. The teacher would hold her hand and keep her on the side while the other children played. Josephine cried as she told me about this and I was heartbroken for her. I was so angry at the teacher, but I realized it was ignorance. I immediately went up to the school to explain to her that they should be careful, but never treat her differently or ostracize her in that way. Thank goodness my husband was able to go to work and I was blessed to be a "stay-at-home" mom which allowed me to be there for Josephine, as well as Gloria, and tackle these kinds of situations head on.

We faced many other challenges, including having to take Josephine from doctor to doctor as a baby. We wanted to find the right doctors to diagnose and treat her. When she had to go for treatments I remember driving long distances to the hospital through blizzards and major traffic jams. We worried about her getting sick from a virus or other infection which could be life threatening as well as putting her through surgery and other medical procedures. It was so heartbreaking to learn that we couldn't even donate blood for her, ever, because we are thalassemia trait carriers. It was very tough to see your child trying to "fit in" with other children, often pretending there was no illness, or her being ostracized because of it. These challenges were very stressful, fearful and frustrating. It's still sad to realize there is no universal cure but I rely on my faith to get me through.

Unfortunately, we didn't find out about the Cooley's Anemia Foundation until Josephine was in her late teens so it was a



Josephine Bila



Michael J LoCurto

challenge for us to not have other parents in the same situation to talk to as we watched Josephine deal with this on her own because she didn't know anyone else with thalassemia. These were the days before we all had computers, smart phones and social media. One other challenge was wondering if we wanted to take the chance on having another child who could possibly be born with thalassemia. We decided to place our trust in God and six and a half years after Josephine, I gave birth to our daughter, Gloria, who miraculously doesn't have thalassemia. She is not even a carrier of the trait. Having a second child presented different challenges as we tried to keep things as normal as possible for Gloria, since attention was usually focused on Josephine's health. We tried to give them equal time and attention as well as make them feel equally loved, healthy and safe. We are proud to say they both grew up to be responsible, caring women of strength, dignity and great courage.

CAF: Your daughter Josephine is on the CAF Board of Directors and is a great advocate for the thalassemia community. You must be very proud of her. What have your greatest joys been parenting a child with thalassemia?

One of the greatest joys is seeing how Josephine could grow and blossom despite all the challenges which not only included having thalassemia but the normal everyday challenges every child must face in keeping up with their peers. Just getting through a school day was sometimes difficult, but she persevered and went on to receive a Bachelor of Arts degree as well as a Master's Degree in Social Work. We know we have done the best we could to make sure she had the best care

and everything we could do to provide as normal a life for her and her sister. We are beyond proud of the woman Josephine has become. Watching her grow from a shy, insecure child into this beautiful, talented, educated and profound human being is an amazing blessing. To see her impact on the thalassemia community around the world is inspiring and brings tears to our eyes. Not only is she a true thalassemia warrior in every sense of the word, she is our hero.

CAF: What made you decide to begin your own Care Walk? Your Care Walk is honoring both your daughter Josephine and the late Michael LoCurto. Could you tell us about Michael and why you wanted to honor him in this way?

I decided to begin my own Care Walk because, having a child with thalassemia, it is a cause close to my heart. I wanted to do something to help bring awareness to the community about the impact thalassemia has on those who have it, especially since most people have never heard of it. I also want to make sure we are getting the info out there in support of the CAF to ensure that every child, parent and family dealing with thalassemia has this important resource to help them. I also feel it is my way of bringing the community together in an important but fun way, while giving back to the thal community and helping those affected. Organizing the Care Walk led me to befriend two thalassemia families up here, the Wagars and the LoCurtos. Michael LoCurto was a Buffalo native who was well loved not only by family and friends, but by the community he served as a Buffalo Common Council Member and Deputy Commissioner of the Erie County Department of Environment and Planning. Michael's parents came out to help with the first two Care Walks with Michael being able to attend one. They were also visitors to our home and our connection through thalassemia makes us all one family. He was a remarkable, brilliant, funny, caring individual who did much to help rebuild his community and the businesses there as well as fight for the underdog. My family admired him very much although we wish we had gotten to know him better. He, like Josephine, went out into the world to make a difference. We always hold the Care Walk in honor of Josephine, as well as Katrina Wagar, but this year we are holding it in Michael's memory, not only because he was a thalassemia warrior, but since his death, thalassemia has been spoken about on all the news channels and in all the articles written about him. His family wants people to know that this blood disorder exists. People need to listen and something needs to be done to find a universal cure. No parent should have to say goodbye to their child whether that child is a toddler, a teen or an adult.

CAF: What is your greatest hope for people with thalassemia?

My greatest hope is that people with they understand that, even though they have thalassemia, it does not have them! We always told Josephine that she can do and be anything she wants to be because nobody is more special than her and thalassemia doesn't define who she is. I hope people with thalassemia do their best to live their best lives and be their best selves despite it, because they are stronger than they know. They are thalassemia warriors!

CAF'S ANNUAL GALA ON JUNE 7 IS A CELEBRATION OF HOPE

CAF is proud to present our annual Gala on Thursday, June 7, 2018 at the Lighthouse at Chelsea Piers in Manhattan. Nick Leschly, chief bluebird of bluebird bio, will accept the Humanitarian of the Year award on behalf of the company, which is conducting clinical trials in gene therapy for beta-thalassemia, as well as developing treatments for other conditions. Tracy Antonelli will receive the Patient Recognition award in acknowledgement of her triumphs over the challenges of living with thalassemia and her adoption of three children with thalassemia from China.

With its lentiviral-based gene therapies, T cell immunotherapy expertise and gene editing capabilities, bluebird bio has built an integrated product platform with broad potential application to severe genetic diseases and cancer. bluebird's investigational one-time gene therapy has the potential to eliminate or reduce chronic blood transfusions in patients with transfusion-dependent β -thalassemia.

In making this announcement, Peter Chieco, incoming National President of the Cooley's Anemia Foundation Board of Directors, said, "For years, the Cooley's Anemia Foundation has been a champion of gene therapy as a potential curative approach to thalassemia. We are thrilled to have this opportunity 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."

"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."

— 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

A NIGHT FILLED WITH HOPE

COOLEY'S ANEMIA FOUNDATION

Cordially invites you to its

2018

ANNUAL GALA

THURSDAY • JUNE 7, 2018
6:00 IN THE EVENING
LIGHTHOUSE AT CHELSEA PIERS

 Cooley's Anemia
FOUNDATION
Leading the Fight Against Thalassemia

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."

Cooley's Anemia Foundation is also honoring Tracy Antonelli, recipient of the Patient Recognition Award. 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.

"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.”

The 2018 Gala Chairman, Frank Fusaro, President, The Forum Group; Chairman, Columbus Citizens Foundation; and Member of the Board of Directors of Cooley’s Anemia Foundation announced, “Your support of our efforts on behalf of our patients and their families is very appreciated. Please join us on June 7th to help us honor these deserving individuals and to meet some of the members of our thalassemia family and many of us who support them. I hope to see you at the Lighthouse at Chelsea Piers at 6:00 on June 7th!”

To RSVP, visit bit.ly/CAFGala2018.

In Memoriam

We regretfully report the loss of Cooley’s anemia patient **MICHAEL J. LOCURTO** and extend our sympathies to his friends and family.

UPCOMING EVENTS

CAF CARE WALK • MAY 6, 2018

Annual fundraising event and opportunity to bring together the thalassemia community and its supporters around the country. Funds raised support medical research to fight thalassemia as well as patient support services for thalassemia patients across the country.

Join Care Walk 2018 at bit.ly/carewalk2018.

CAF ANNUAL GALA • JUNE 7, 2018

RSVP at bit.ly/CAFGala2018.

CAF 2018 PATIENT-FAMILY CONFERENCE JULY 6–8, 2018 • ATLANTA, GEORGIA

Contact sgilbert@thalassemia.org if you wish to be put on a list to receive additional information when it becomes available.

Register by June 12 at <http://www.docbw.com/meet/caf/>.

SANGAMO THERAPEUTICS AND BIOVERATIV THERAPEUTICS BEGIN NEW CLINICAL TRIAL FOR TRANSFUSION-DEPENDENT BETA-THALASSEMIA

Sangamo Therapeutics and Bioverativ Therapeutics have begun a Phase 1/2 study of gene editing in patients with transfusion-dependent beta-thalassemia in the U.S. This trial will investigate whether increasing the production of fetal hemoglobin can reduce or eliminate the need for blood transfusions. Participating patients will have their CD34+ blood stem cells removed and genetically edited in order to boost their production of fetal hemoglobin (HbF). This process is completed without the use of integrating viral vectors. These edited stem cells will then be infused back into patients after receiving conditioning chemotherapy to make room for the new cells in the bone marrow. The trial will enroll 6 patients between the ages of 18 and 40 with transfusion-dependent beta thalassemia. The first trial site has just opened in Oakland and additional trial sites are expected to open soon. Further information is available at:

<http://bit.ly/SangamoBioverativeTrial>

The Cooley’s Anemia Foundation provides information on select clinical trials that may be of interest to the thalassemia population. This information is provided for educational purposes only and does not imply an endorsement of any trial. Patients who are considering participating in a clinical trial and do not know what questions to ask may want to consult with CAF Patient Outreach Director Sandy Gilbert (sgilbert@thalassemia.org) who can help them determine what questions they should ask the investigators to determine if a trial is right for them.

Do you have a Primary Care Provider?

Most people with thalassemia need a Primary Care Provider (PCP) to treat non-thalassemia related issues.

Your hematologist can collaborate with your PCP about how thalassemia might affect your general healthcare needs.

CARE WALK 2018 BRINGS THOUSANDS TOGETHER IN CITIES ACROSS THE U.S. (CONTINUED FROM COVER)

CARE WALK 2018 TEAMS:

Bae Needs Blood	Mustang Sally	Team Ercole
Brave Buckeyes	North Jersey	Team Ficarra
Buffalo Cares	StL Thal Pals	Team Giuseppe
Chicago Care Walk	Team A.J.	Team Joshie
Cooley's Kids	Team Acceleron	Team Jovi
DC Chillin'	Team Bhanu	Team Minnesota
Kayla's Care Walk	Team Candace	Team Ruby
In Loving Memory of Michelle	Team Corbyn and Cai	Team Staten Island
LA Dreambuilders	Team DNA	Team Zayna
La Jolla Pharmaceutical	Team Doherty	The A Team
Mia's Marchers	Team Emily Grace	#teamdailey

GOLDEN LEADER SPONSOR

\$25,000 LEVEL



BRONZE POWER SPONSORS

\$7,500 LEVEL



FRIENDS OF CAF



UFOLI (7-ELEVEN) ADOPTS CAF AS ITS OFFICIAL CHARITY

This past year, the 7-Eleven United Franchise Owners of Long Island and New York unanimously adopted Cooley's Anemia Foundation as their official charity of choice. This means that 7-Eleven stores on Long Island volunteer to put donation boxes near their cash registers to collect spare change for CAF. This has already generated almost \$17,000 since collections began in June of 2017. In addition, the UFOLI Board of Directors decided to give CAF a separate donation of \$5,000 at their annual Holiday Party.

We are grateful to UFOLI and its members for their generosity. We are also most grateful to Michael Pouchie and Michael Presto, CAF Board Members and co-owners of National Distribution Alliance, the premier newspaper and magazine distribution company that serves, among others, the 7-Elevens of Long Island and New York. Mike and Mike and their colleagues at NDA make daily deliveries to all the UFOLI franchises and it was their initiative that caused the UFOLI Board of Directors to consider Cooley's Anemia Foundation as their primary charity.



The UFOLI Board of Directors presented CAF with a check (number 711) for \$5,000 at their annual Holiday Party on December 11, 2017. Pictured left to right are **Basit Khurshid**, UFOLI Sr. Vice President; **Henry Ackermann**, CAF National Development Director; **Yousaf Naseer**, UFOLI President; **Michael Pouchie**, President & COO, National Distribution Alliance LLC and CAF Board Member; and **Nasir Ahmad**, UFOLI Treasurer.

Mike and Mike also volunteered to help CAF by serving as the hands-on overseers of the collection process. NDA staff provide the collection boxes, empty the contents, and give the proceeds to CAF. This is a terrific example of volunteers going out of their way to help our patients and families.

WITH YOUR HELP, THE CURE IS WITHIN REACH!



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

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

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

NAME _____

ADDRESS _____

CITY/STATE/ZIP _____

E-MAIL _____

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

MASTERCARD VISA AMEX

CARD # _____ EXP. _____

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

All contributions are tax-deductible.

A LOOK BACK ON CARE WALK 2017

