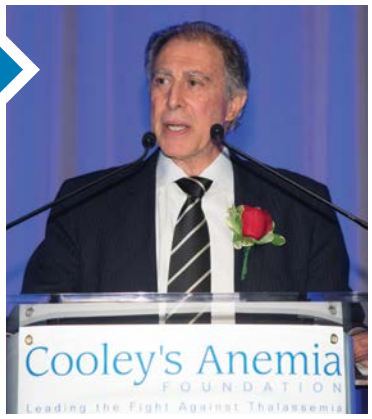
WINTER 2017

LIFELINE

COOLEY'S ANEMIA FOUNDATION • LEADING THE FIGHT AGAINST THALASSEMIA

SPINO AND TRICTA HONORED FOR ROLE IN BREAKTHROUGH DRUG



Dr. Michael Spino (top) and Dr. Fernando Tricta (left) accepting their Humanitarian of the Year awards.

Two men who championed a life-saving drug for patients with thalassemia were honored at the annual Cooley's Anemia Foundation Gala on June 8th, in New York City. Drs. Michael Spino and Fernando Tricta jointly received the Humanitarian of the Year Award for their work on deferiprone (Ferriprox™), an oral medication used to treat iron overload caused by blood transfusions in people with certain hereditary red blood cell disorders (thalassemia syndromes).

Prior to the approval of Ferriprox™, many patients died from cardiac failure and other complications from iron overload, as well as the inadequacy of the only existing treatment. Patients also suffered a significant burden on their quality of life due to the need of a treatment process that required lifelong, daily 8-12 hour injections to remove excess iron from the body.

Anthony Viola, National President of the Cooley's Anemia Foundation Board of Directors, announced, "Doctors Spino and Tricta are true heroes in the thalassemia community. Their efforts have led directly to prolonging the lives of our patients and enhancing the quality of their lives. We are so grateful that they have agreed to let us publicly acknowledge them."

"It is humbling to have this honor bestowed on us, but the impact that this drug has had on so many lives would not have been possible without the unwavering support of Dr. Barry Sherman, the Founder of Apotex," said Dr. Spino. "To this day, we still receive personal, touching stories from patients living with thalassemia, telling us how this drug has positively changed their lives."

Co-recipient of the award was Dr. Fernando Tricta, a pediatric hematologist who was instrumental in the clinical studies for the assessment of the safety and efficacy of deferiprone in his pursuit of better treatment options for people with thalassemia. Almost 20 years after its first approval, he still reflects on his first experiences with the drug. "When I first started treating patients with this drug on a compassionate basis, I could not believe the improved quality of life it had versus the available treatment at the time. More recently, deferiprone was recognized by the American Heart Association for its effectiveness in removing excess iron from the heart, which was the main cause of death in transfused patients with thalassemia."

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CAF INTERVIEWS DR. TIPPI MACKENZIE ON FIRST EVER CLINICAL TRIAL INVOLVING IN UTERO STEM CELL TRANSPLANTATION FOR ALPHA-THALASSEMIA MAJOR (ATM)

The University of California, San Francisco (UCSF) is currently recruiting participants for the first ever clinical trial performing in utero stem cell transplantations on fetuses affected with alpha-thalassemia major (ATM). CAF speaks with Dr. Tippi MacKenzie, a pediatric and fetal surgeon at UCSF Benioff Children's Hospital who is leading this clinical trial as the principal investigator.

CAF: How severe is a prenatal diagnosis of alpha thalassemia major (ATM)? What percentage of these fetuses survive without treatment?

TM: Alpha thalassemia major is almost uniformly fatal in utero without intervention. These fetuses develop severe anemia which results in a condition called "hydrops" that includes heart failure.

CAF: What treatments are currently available to allow fetuses diagnosed with ATM to survive and be born? How effective are these treatments? What complications are associated with these treatments?

TM: When a prenatal diagnosis of ATM is confirmed, pregnancy management options include close fetal monitoring, pregnancy termination, or fetal intervention with in utero transfusions. These transfusions are designed to treat the fetal anemia and can result in the birth of a healthy baby with good neurological outcomes. However, the surviving patients will continue to require lifelong blood transfusions, or a stem cell transplant. Our group is now starting a clinical trial of combining in utero transfusions with a stem cell transplant with the hope that this will become a single, definitive treatment.

CAF: Your phase 1 clinical trial is currently recruiting participants and will demonstrate the safety, feasibility and efficacy of performing in utero stem cell transplantation on fetuses affected with ATM. The maternal participant will undergo bone marrow harvest. The harvested maternal stem cells will then be transplanted into the fetus via an in utero transfusion (IUT). Is this correct?

TM: Yes, that is correct. The stem cells are harvested from the mother because the fetus will tolerate the mother's stem cells during pregnancy and will therefore not require any immune suppression.

CAF: What are the expected benefits of IUT of stem cells and how do these benefits compare with the benefits of IUT of red blood cells?

TM: In utero transfusions of red blood cells are a short-term measure to correct the fetal anemia. They have been successful in protecting the fetus from the effects of anemia until birth. However, babies born after transfusions will still require lifelong therapy with continued blood transfusions, or a stem cell transplant. The in utero stem cell transplantation, if successful, could result in a more definitive therapy since the patient could then make his or her own functioning red blood cells from the transplanted stem cells. Even if there are not enough surviving stem cells to fully replace all red blood cells, the patient can get a "booster" stem cell transplant after birth, which is usually safer than a brand-new stem cell transplant.

"For many years, patients with alpha thalassemia major were not given any options other than pregnancy termination... We are excited to offer this new therapy of combining the transfusions with a stem cell transplantation since it offers the possibility of a more definitive therapy."

– Tippi MacKenzie, M.D.

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

Dear Friends:

It is with an extremely heavy heart that I am announcing that I will be stepping down as National President effective the next National Board of Directors meeting in May, 2018. My very strong recommendation and endorsement for my successor is former past National President Peter Chieco, as Peter has agreed to be considered for the role as the next National President. I could not think of anyone more qualified to take on this position to lead the Foundation going forward.

My reasons are simple:

- 10 years is a long time, and I felt like my "message" was getting stale. I have felt this way for a while now and my feelings on this have been proven to be correct. No one person is bigger than the Foundation.
- I can no longer fulfill the role of National President in the way that I would like to. I have a very high standard for properly representing this great Foundation and that means being visible as much as possible. Unfortunately, this takes a physical toll after a while.
- I have accomplished my goals that I originally stated in my first agenda back in 2008. Please see the Foundation's accomplishments during my tenure listed below.
- The right person is willing and able to effectively take on this role, so overall, the timing is right. Also, and most importantly, Peter has a much better grasp on the medical side than I do. The next 2-5 years there will be significant medical breakthroughs and Peter is much better qualified than I am in this area.

My role with the Foundation going forward will be as follows:

- I would request for my successor to name me the National Cooley's Chair for OSIA, UNICO, UFOLI, The Milana Family Foundation, and the other organizations the Foundation has a relationship with.
- I will continue to sit on the National Board of Directors.
- I will continue to sit on the Gala Committee and I will continue to have a substantial role in the Foundation's fundraising.

The following is a list of a few of the Foundation's accomplishments during my terms as National President:

2008-2017

- CAF has awarded more than $3,300,000 in Fellowships and Research Grants to researchers and physicians as recommended by our Medical Advisory Board. This includes funds for our Gene Therapy/Stem Cell Transplant Research Grants, which originated during this period.
- Since 2008, Ferriprox ("L1"), Exjade for non-transfused dependent patients, and Jadenu have been made available for thalassemia patients for their daily treatment regiments.
- The CAF registry of patients increased from 700 to more than 1100 names since 2008.
- In association with the New York Academy of Sciences. CAF has held 2 very successful Cooley's Anemia Science Symposia (2009 and 2015), bringing in top thalassemia experts from around the world to report on their progress in assisting thalassemia patients.
- CAF has held 6 Patient-Family Conferences (2012 to 2017) at which attendance has grown from 200 to 335.
- CAF has raised more than $18 million in total funds between July, 2008 and June, 2017.
- The CareWalk increased from 1,000 participants to 3,000 participants, increasing its donations from $150,000 to $311,500.
- The Annual Gala Dinner Dance, a National fundraising event, was initiated in 2014 to replace the Cigar Night with the intention of drawing more of a donor base with a bigger event than the Cigar Night. It has raised almost $1,000,000 in four years.
- UFOLI, an organization of 7-11 franchise holders in Long Island, generously adopted CAF as its corporate charity, placing donation boxes in hundreds of stores.
- The Milana Family Foundation has named CAF as a beneficiary of two high-profile golf outings which have raised more than $150,000 combined for CAF.

Thank you for your support and love over the years. You are all like family to me and I have treated this responsibility as such. The thalassemia community is such a special group of people that rally and encourage each other. It inspires all of us at the Foundation to do the very best we can and do even more. Your life successes and accomplishments are the reward the Foundation asks for. Keep moving forward and please use the Foundation whenever needed. We are here to serve you, our clientele.

I look forward to seeing you all soon, as I am excited to attend the Foundation's Patient Conferences in the coming years. Please stay well and please to continue to thrive with thal.

With love,
Tony Viola

RECORD-BREAKING ATTENDANCE AT THE CAF 2017 PATIENT-FAMILY CONFERENCE

See additional photos on back cover.

The 2017 CAF Patient-Family Conference was a true success, with a record-breaking attendance of over 300 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. Next year's Conference will take place from July 6-8 in Atlanta, GA. We hope to see you there! For more information, visit www.thalassemia.org or email info@thalassemia.org.

SAVE THE DATE!

COOLEY'S ANEMIA FOUNDATION

CARE WALK
MAY 6, 2018

CAF PROVIDES SCHOLARSHIPS FOR TIF INTERNATIONAL CONFERENCE

14TH INTERNATIONAL CONFERENCE ON THALASSAEMIA & HAEMOGLOBINOPATHIES AND 16TH TIF INTERNATIONAL CONFERENCE FOR PATIENTS & PARENTS

The Thalassaemia International Federation (TIF) held the 14th International Conference on Thalasseamia & Haemoglobinopathies, as well as the 16th TIF International Conference for Patients & Parents at the Grand Hotel Palace in Thessaloniki, Greece from November 17-19, 2017. The Conference was organized in collaboration with Greek Thalassaemia Federation (EOTHA) and the Greek Ministry of Health. As with all TIF regional and international conferences, the TIF International Conference was comprised of two parallel programmes – a Scientific Programme for Medical Specialists and another for Patients/ Parents.

The Cooley's Anemia Foundation supported the attendance of 8 U.S. thalassemia patients and parents of thalassemia patients at this Conference through a scholarship fund in order to help defray some of the costs associated with traveling to and attending the Conference. We applaud TIF for hosting yet another very successful conference and for bringing together the global thalassemia community. To learn more about TIF and their work, visit www.tifevents.org.

THALASSAEMIA INTERNATIONAL FEDERATION

CAF ACCEPTING APPLICATIONS FOR 2018 MEDICAL RESEARCH FELLOWSHIPS AND GRANTS

The Cooley's Anemia Foundation is accepting applications for medical research grants and fellowships in areas related to thalassemia. The awards are in 3 categories: Support for Ongoing Clinical Research in Thalassemia; Clinical Trials in Thalassemia Cell and Gene Therapy; and Research Fellowships. To download applications, visit www.thalassemia.org or email info@thalassemia.org.

Details are as follows:

SUPPORT FOR ONGOING CLINICAL RESEARCH IN THALASSEMIA ($40,000)

The Cooley's Anemia Foundation invites national and international applicants to apply for grants to support ongoing clinical research projects in thalassemia.

The goal of this initiative is to support investigators from all disciplines and backgrounds (MD, RN, PhD, MPH, MSW or other disciplines) with their ongoing clinical projects to address one or more of the following areas impacting patients with thalassemia, including but not limited to: cardiac issues and iron overload; fertility, pregnancy and family planning; quality of life, psycho-social impact and/or burden of disease.

CLINICAL TRIALS IN THALASSEMIA CELL AND GENE THERAPY GRANT AWARD ($60,000)

The Cooley's Anemia Foundation invites national and international applicants to apply for grants to facilitate clinical trials in Cell and Gene Therapy to advance a cure for thalassemia. Both phase I (safety) and phase II (efficacy) trials are eligible for support.

RESEARCH FELLOWSHIPS ($32,500)

The Cooley's Anemia Foundation invites national and international clinical and basic science investigators (postdoctoral or junior faculty) to apply for its prestigious fellowship program. Applications should be focused on the understanding or treatment of thalassemia or a complication that is related to thalassemia. The areas of interest include, but are not limited to, studies of globin gene regulation, globin gene transfer and expression, fetal hemoglobin production, hematopoietic stem cell research, bone marrow transplantation, iron chelation and iron overload, endocrine and cardiac disorders in thalassemia, and transfusion therapy and its complications.

JADENU SPRINKLE GRANULES NOW AVAILABLE THROUGH SPECIALTY PHARMACIES

JADENU Sprinkle (DFX) granules are now available. These granules are intended to be sprinkled on soft food (eg, yogurt or applesauce) immediately prior to use and administered orally. JADENU Sprinkle contains the same active ingredient as EXJADE.

The Cooley's Anemia Foundation provides information on thalassemia treatment options that may be of interest to the thalassemia population. This information is provided 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 treatment.

CAF ACCEPTING APPLICATIONS FOR 2017-2018 PATIENT INCENTIVE AWARDS

The Cooley's Anemia Foundation is offering incentive awards for thalassemia patients to further their education and career goals.

The deadline to apply is February 15, 2018. No extensions will be granted, so be sure to apply by the deadline. Awards will be distributed in March, 2018. To download the application, visit www.thalassemia.org or email info@thalassemia.org.

THE INCENTIVE AWARDS WILL BE GIVEN OUT AS FOLLOWS:

- $2,000 will be awarded to students enrolled in Doctorate programs. Patients may receive two postgraduate level awards over the course of their studies.
- $1,500 will be awarded to students enrolled in Master degree programs. Patients may receive two graduate level awards over the course of their studies.
- $1,000 will be awarded to students entering or continuing full time studies towards a Baccalaureate degree. Patients may receive four undergraduate level awards over the course of their studies.
- $750 will be awarded to students entering or continuing full time studies towards an Associate degree. These are limited to two per applicant over the course of his/her pursuit of an Associates degree.
- $500 will be awarded to students enrolled in certificate programs lasting for one year or longer. These awards are limited to two per applicant.
- $250 will be awarded to students enrolling in six-month certificate programs or vocational training programs. These awards are limited to two per applicant.

Please note: the amount of your award cannot exceed the cost of your tuition.

SAGE BIONETWORKS LAUNCHES JOURNEY PRO APP

Sage Bionetworks, a non-profit biomedical research accelerator, together with Celgene Corporation has announced the launch of **Journey PRO**, a mobile health research study designed to improve the understanding of disease burden on people living with chronic anemia due to myelodysplastic syndromes (MDS), myelofibrosis, and beta-thalassemia.

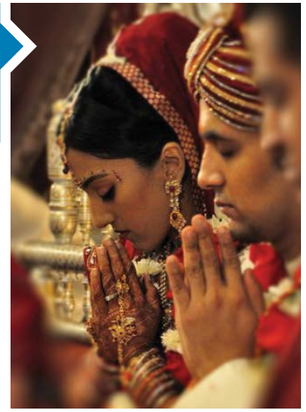
This study uses mobile and wearable technologies to quantify the daily burden of chronic anemia on patients living with these disorders. The study utilizes the Apple ResearchKit framework to collect data from participants using surveys, neurological self-assessments using the BrainBaseline cognitive testing software from Digital Artifacts, health data collected from the iPhone HealthKit, and fitness tracking wearables, among others. By following participants using these quantitative assessments, the aim is to develop a tool to evaluate the efficacy of new treatments for reducing the impact of these disorders on patients.

The Journey PRO app was designed with input from members of the chronic anemia community to support patients in the management of their health. Participants may use the app to track key health data (e.g., transfusions, lab values) and the scheduling of appointment dates. Participants will also be able to visualize their study data and may choose to download the data and share it with their healthcare team. This design was developed in consultation with patient representatives recruited through the MDS Foundation Cooley's Anemia Foundation, and the Leukemia and Lymphoma Society.

Journey PRO is open to participants over the age of 18 living in the United States with an iPhone model 5 or newer with iOS 8 or later. The research term encourages patients with a diagnosis of myelodysplastic syndromes (MDS), myelofibrosis, and beta-thalassemia to participate in this study. Individuals without a diagnosis of chronic anemia can also participate by providing valuable comparison data to further illustrate the difference in quality of life measures between the chronic anemia population and the general population. The Journey PRO app is available immediately for download from the App Store.

VOLUNTEER SPOTLIGHT

CAF launched our "Volunteer Spotlight" Series last year 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 heartfelt 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.



WELCOMING THE RAINBOW BABY: THE STORY OF A PHILADELPHIA COUPLE'S JOURNEY TO PARENTHOOD

Doctors Monisha and Parag Vora are an inspiring couple who are both beta thalassemia carriers and are currently expecting a child. They have decided to forego a traditional baby shower with registry and gifts, and instead launched an online baby shower fundraiser to support thalassemia medical research. CAF thanks Monisha and Parag for sharing their story with the community through this interview, and for celebrating their baby girl by helping individuals with thalassemia. We wish you the very best on your journey to parenthood!

"We encourage all couples who are carriers to share their stories as this is what provided us hope, and we hope that our story may provide hope for other couples out there who may be going through a similar experience."

– Parag Vora, M.D.

Parag Vora, M.D. and Monisha Vora, M.D.

CAF: Where do you live and what are your occupations?

Monisha: We live in the City of Brotherly Love: Philadelphia, Pennsylvania. I am an ophthalmologist in private practice just outside the city.

Parag: I am a physician who takes care of kids in the emergency room.

CAF: Could you tell us a little about your story together?

M: We met in August of 2006 at a wedding in Cleveland, Ohio. At the time, Parag was a third-year medical student at Case Western Reserve University and I was just starting my first year of medical school at Stony Brook University in New York. We got engaged in the summer of 2009 and then married one year later in 2010.

P: That's right. The best thing to come out of my life (so far) started in Cleveland, Ohio, which is something that you probably don't hear too often. I like to joke that we met at a bar (because that was the first place the group that we were both with hung out together in pre-wedding festivities). Not too long after the wedding, we started chatting online, over e-mail, and on Skype, and eventually upgraded to the phone. I made my first visit to Long Island that October. As a student on a limited budget, I initially endured many long car trips and overnight bus and train rides. Two years later, I matched for my pediatric residency on Long Island, and the rest, as they say, is history!

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

M: I knew I was a beta-thalassemia carrier since medical school when routine blood work revealed that I was slightly anemic. With a known family history of the trait on my father's side, I later had confirmatory testing that revealed I was a carrier.

P: I had a vague recollection that I had been diagnosed as a carrier, and knew it ran in the family on my mother's side. I had confirmatory testing in 2015, only to later find records from the late 1970s or early 1980s that confirmed it already.

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This year, CAF began a new annual tradition of recognizing National Adoption Month throughout the month of November. Our goals are to bring attention to the need for permanent families for children with thalassemia both in the U.S. and globally, to provide the greater community with resources and information on thalassemia adoption, and to share the stories and perspectives of adoptive families. We hope you are inspired by their journeys!

HOW SIGNIFICANT IS THALASSEMIA COMMUNITY ADOPTION IN THE U.S.?

At the current time, 12% of thalassemia patients in CAF's U.S. Patient Database have been adopted from other countries. The vast majority of these adoptions come from China, although there are families that have adopted children with thalassemia from Vietnam, India, Afghanistan, Greece, Albania and other countries. Many of these families have adopted more than 1 child with thalassemia: 21 of the adoptive families we are in contact with have adopted 2 children with thalassemia, 3 families have each adopted 3 children with thalassemia, and one family has adopted 4 children with thalassemia.

HISTORY OF NATIONAL ADOPTION MONTH

National Adoption Month has been recognized in the U.S. since 1995. It began as a White House initiative in collaboration with of the Department of Health and Human Services (HHS) to promote the use of the internet to match children in foster care with adoptive families. Over the past two decades, this initiative has contributed to the growth of a strong network of adoption communities throughout the country. CAF is proud to be a part of that network, and we are committed to providing resources and support for the thalassemia adoption community.

Visit www.thalassemia.org/adoption for more interviews with adoptive families, FAQs about adoption, and resources available to those going through the process of adopting a child with thalassemia.

RAISING BLOOD BROTHERS: A FLORIDA FAMILY'S JOURNEY WITH THALASSEMIA AND ADOPTION

From left: Cai, Callie, and Corbyn

CAF thanks the Cammilleri family for sharing their story with the community. We hope you are inspired by their journey!

Could you tell us a little about yourself and your family?

My husband Joe and I live in Florida along with our three blessings, our children Corbyn (9), Cai (8) and Callie (7). We met in pharmacy school and he is now a full time clinical pharmacist and I am a part-time retail pharmacist.

When did you decide that adoption was right for your family? What made you make this decision?

It's a really long God-filled story, but I'll try to keep it short! Joe and I discussed adopting when we were dating. We went on some medical mission trips where we were also able to spend some time in orphanages. This stirred several conversations of one day hoping to adopt. When our first born was diagnosed with thalassemia major, we were already pregnant with our daughter. We found out she did not have thalassemia when she was tested at birth. Managing our son's disorder while also caring for an infant was very challenging. But still, we had this feeling that we were not done. We prayed about the path we should take to grow our family and we felt called to adopt a child living with thalassemia who otherwise may not be able to receive proper care.

We reached out to CAF who put us in touch with a group who listed waiting children's files by disease severity through a special needs program. We soon learned that China had a severe blood shortage and children were suffering and not surviving as a result of not receiving the treatment they needed. When we saw our son's face, we knew he was meant to be in our family. We began the steps to adopt him and he joined our crazy crew in 2013 at four years old!

The Cammilleris excitedly await Cai's arrival. From left: Corbyn, Lynzie, Joe and Callie

How did you learn about thalassemia?

We learned about the depth of thalassemia when our son, Corbyn, was diagnosed as a baby. I was aware that I was a carrier of thalassemia (minor) but we did not know my husband was. We did genetic testing before we attempted to get pregnant and were told he was not a carrier. When we were trying to figure out Corbyn's diagnosis, my husband was retested and was shown to, in fact, be a carrier as well. There is a 25% chance that two parents who carry the defective gene will have a child with thal major. Our two boys both have the major form of thalassemia, also known as Cooley's anemia.

Tell us a bit about your road to adoption. How long was the process? What were some of the challenges you faced?

Our journey to our son was fairly quick! We went through a special needs program and children who have thalassemia are typically expedited because of their pressing need for transfusions to stay alive. From starting our research to having him in our arms was about 7 months! I was fortunate to join an adoption support group full of parents who helped me with paperwork and preparing for the road ahead.

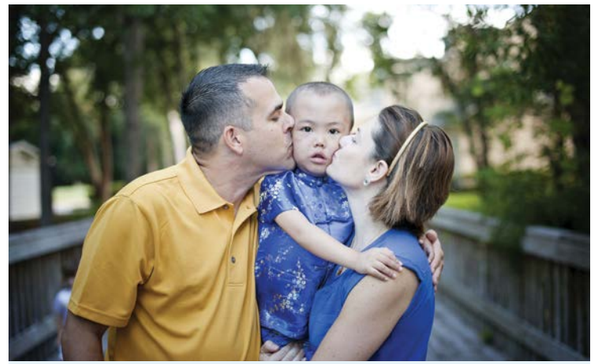
Adoption comes with many obstacles, but anything that's worth it does, right? For me, the first was waiting to go get our son. Not knowing if he was being loved on and his medical needs were being met was difficult for me. I cried a lot of tears praying he'd make it until we got to him.

When you're pregnant, you are in control of making sure you are healthy and making the best decisions for your child. When you're in the adoption paper chase, decisions about your child's well-being are out of your control. Throw in a severe medical need, and it's even scarier. Adoption was by far the worst "labor pains" I have ever experienced. The moment I had that boy in my arms (screaming albeit!) gave me the biggest sigh of mama relief. There are long term challenges like attachment, loss and trauma that will continue to come and go due to circumstances out of his control. The good news is that he will always have a family that loves him through everything, no matter what.

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

For us, our biggest concern was expense. We already knew what it was like to parent a child with thalassemia. We knew this road would not be easy medically or emotionally. We also knew how costly caring for one child with thal was, so the costs of international adoption in addition to ongoing medical costs for two chronic blood disorders seemed overwhelming.

But we believed that God put this in our hearts and when He brings you to it, He will bring you through it! Other initial fears were that our adoptive son might have irreversible damage from not getting the treatment he needed, additional medical needs not listed in his file, and possible delays related to what he had gone through. Thankfully, none of these were deal breakers for us!



Finally home! Cai with parents Joe and Lynzie

Parenting a child who has been through such loss can be hard. Saying the right things and making the right choices are fears, but the same issues come up with our biological children as well. We can just do the best we can with the knowledge we have and let all of our children know that they are meant to be in our family, are valued and we will do our best to keep them safe and know they are loved more than anything else in the world.

How did you find answers to your questions about adopting a child with thalassemia?

After we received approval from China to adopt Cai we joined an adoption group that was and continues to be a huge source of support. Since this disease is rare and we are spread out all over the country it's wonderful that we are able to connect and help one another through social media. Since then, I also run a private Facebook group for people parenting thalassemic children. We are a small group, but we are tight knit.

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The blood brothers. From left: Cai and Corbyn

What are the greatest joys of being an adoptive parent to a child with thalassemia? What are the biggest challenges?

Having a child with thal has completely changed our outlook on life as parents. We slowed down, we appreciate every single second. To be honest, my greatest joys are the same with my adoptive son as my biological children. My heart bursts with joy when I get to see his "firsts," when he shares his day, snuggles me and tells me he loves me more than anything. The fact that I get to be his mom is one of my greatest joys. Adopting Cai has also brought me into a deeper relationship with Jesus. It's made me realize how much He loves me (because He has adopted me!) and how all of His children are important to Him. It's such a joy to have a front row seat to his miracles!

The biggest challenge for me is watching my thal babies go through really hard medical things and feeling helpless. Some days there is needle stick after needle stick. When they can't get a vein, we don't have a choice. They NEED a transfusion. There are procedures and sedation and the risks that are read out loud to you time after time. You sign the papers and you keep putting on a brave face for them like everything will be ok and it's just "routine." But your mama heart is crushed every time you hold them when they cry through the physical and emotional pain of thalassemia. I just wish I could take it away. But it is not going away. This is their life. And on the other side of that is THIS IS THEIR LIFE! We build thal into our routine. We go to school, we get labs, we play outside, we get a transfusion, we have dinner together and we get to live this lucky life with them. We take it one day at a time and we focus on our blessings. Thalassemia takes a huge toll on the entire family, but it is also a HUGE blessing. I believe our boys are stronger, more resilient, more caring and empathetic and will one day be world changers.

For me, advocating for their needs is another challenge. Fighting for your child to get the best care possible can be hard when you don't always get to call the shots. Their hematologist may disagree on a certain path of care which can be hard when things are constantly changing. We make it a priority to educate ourselves on current treatments and share that with our medical team. We also try to stand beside their teachers in understanding their medical needs. And we can't forget fighting with the insurance company to get necessary prior authorizations month after month. There are just so many areas to advocate! We are at a new point where we are trying to teach them to be their own advocates. It's a tough gig.

CAF: How did you learn about Cooley's Anemia Foundation, and what makes you support CAF?

When our oldest son Corbyn was diagnosed, we did our research and found CAF! They have truly been a light in our life. They have helped with finding specialists, being a constant source of support, financial assistance, and the list goes on.

Many people at CAF are not just contacts, they are friends. I cannot tell you what CAF has meant to our family, especially before social media when we felt very alone in this disease. We host a Care Walk every year to raise awareness of thalassemia and our goal is to fundraise for CAF to support the work they do for us and others living with thalassemia. They help fund major research and we are progressing rapidly in care because of their efforts. They are my go-to thal experts and, most importantly, they brought us to our son and I am forever grateful.

"Thalassemia takes a huge toll on the entire family, but it is also a huge blessing. I believe our boys are stronger, more resilient, more caring and empathetic and will one day be world changers."

– Lynzie Cammilleri

Corbyn and Cai on transfusion days

Callie, age 7

What advice do you have for individuals who would like to adopt a child with thalassemia?

If you already have a child with thalassemia, I would say that it is a huge blessing to have someone for them to go through this with. Having someone to complain to and support you who really gets it is priceless. When we first brought our son home from China, watching Corbyn do medical things first calmed him. If he could see his big brother do it, he could too! We dubbed our boys the blood brothers and it has truly strengthened their bond. No one else in the family will ever get what they go through on their level.

If you are contemplating adopting this special need and it's new to you, don't be scared! It's overwhelming at first, but you will get in a groove and you have so much support now days. These children are worth it!

Some important logistics I believe you should look into before you pursue adopting a child with a chronic disease such as thalassemia are your insurance coverage options, the distance between where you live and a transfusion clinic/hospital, and your career flexibility. Things come up last minute with thal, transfusion schedules change and I believe it's important to be in a job that supports and understands that. Also, you have to understand that your child could have other needs besides thalassemia. Multiple diagnoses are difficult to juggle, BUT AGAIN they are worth it and you wouldn't think twice if your biological child was born with a special need. You're a mom, you do it!

I there anything else you would like to share with the community?

I just want to share that our own supportive community matters! I am so thankful for our thal community, but our community of family and friends supporting us day in and day out is so important, too.

We are extremely grateful when you hold our hands, celebrate our victories, listen to us cry and show up for us. I am continuously amazed at how great our friends are. Everyone who bring their kids to our annual Care Walks. Our friends who explain thalassemia to their children. Our family babysitter who helps us juggle appointments. And their teachers who provide an inclusive environment with their peers.

I also just cannot share about my brave boys without recognizing their supportive sister! Special needs siblings are, well, super special. Sister is always at their transfusions, long appointments, getting them drinks and helping them in any way she can.

In our home, adoption means love and thalassemia means life!

SCIENTISTS HONORED FOR ROLE IN BREAK-THROUGH DRUG (CONTINUED FROM COVER)

The Cooley's Anemia Foundation also honored Dino Philippou, recipient of the 2017 *Young Leadership Award*, an exceptional young man who has become not only a successful restaurateur but also an inspiring and untiring philanthropist and activist for the Foundation and other causes. Mr. Philippou also happens to be a thalassemia patient.

According to Mr. Viola, "Dino is a great benefactor and friend of Cooley's Anemia Foundation. The fact that he is also a patient is almost coincidental and we would honor him anyway because of his support for our cause. We are happy to acknowledge him with this award."

Dino Philippou said, "I am so happy to accept this recognition from Cooley's Anemia Foundation because they have been so instrumental in improving the lives of all of us with this disorder."

The 2017 Gala Chairman, Frank Fusaro, President, The Forum Group; Chairman, Columbus Citizens Foundation; and Member of the Board of Directors of Cooley's Anemia Foundation served as the evening's emcee. The event, attended by nearly 300 people, raised more than $335,000 in support of the work of the Foundation.

The 2017 Young Leadership Award honoree Dino Philippou.

President and CEO of Apotex Jeremy Desai, Cooley's Anemia Foundation National President Anthony Viola, Susan Viola, Founder and Chairman of Apotex Dr. Barry Sherman, Honey Sherman, Olga Spino, Honoree Dr. Michael Spino, Pat Kay, Vice Chairman of the Board of Apotex Jack Kay, Rosa Maria Tricta, and Honoree Dr. Fernando Tricta

Paul Tocci, Kathleen Tocci, Rose Ann Chieco, and Member of the Gala Committee and Member of the Board of Directors and past National President of Cooley's Anemia Foundation Peter Chieco

Member of the Gala Committee and Member of the Board of Directors (and representative of the Board to the Thalassemia International Foundation) and past National President of Cooley's Anemia Foundation Robert Ficarra, and Marianne Ficarra

Member of the Board of Directors of Cooley's Anemia Foundation and thalassemia patient Maria Hadjidemetriou thanking the Humanitarian of the Year awardees for saving her life with their breakthrough medication

(Right) Attendees at the Cooley's Anemia Foundation 2017 Annual Gala cocktail hour making bids on the Silent Auction items

National President of Cooley's Anemia Foundation Anthony Viola presented the awards to the honorees

Founder and Chairman of Apotex Dr. Barry Sherman introduces the 2017 Humanitarian of Year awardees

VOLUNTEER SPOTLIGHT (CONT. FROM PG. 7)

CAF: You must be so excited for your new addition! When are you expecting the arrival of your little one?

M: We are expecting our little girl in early January 2018. She is our rainbow baby and we both feel incredibly blessed.

P: Yes!! I, though, am expecting her to know to arrive in December, slightly earlier than scheduled, so she can be our most precious "tax deduction." I may or may not have Monisha do extra laps around our apartment starting in mid-December. As I turn 40 in mid-December, it will be difficult to plan a party as we don't know when our little one will arrive, but her arrival will be a better present than any party!

CAF: What made you decide to have an online fundraiser for CAF as part of your baby shower?

M: After enduring two difficult back-to-back pregnancy losses in less than 18 months, the second of which was affected with beta-thalassemia major, we initially were not sure if we even honestly wanted to have a shower. However, after finding out that our third (and current) pregnancy was not affected, we felt incredibly blessed and lucky. We wanted to honor our first daughter, Asya (meaning "grace"), who was affected, by raising awareness about the disease and supporting the thousands of children and young adults and their families whose lives have been affected by beta thalassemia major.

P: After our experience, we obviously knew the cause that we wanted to support but weren't aware of the organization until we searched online. In lieu of a registry and gifts, we decided to have our friends and family contribute toward the funds that we will be donating to CAF in Asya's name.

CAF: Why do you support the work of CAF?

P: With all of the research that CAF supports, we sincerely hope that one day in the not too distant future, couples like us will not have to worry about their children being afflicted with Cooley's anemia. Although we know that there will be others like us or that have been even more unlucky than us, we wish that no one would have to go through that experience.

Drs. Monisha and Parag Vora at their wedding

CAF: Do you have any advice to give couples who are beta thalassemia carriers?

P: Although it felt at times like the odds were stacked against us, we had to remember that as 2 carriers, we still had a 75% chance of having an unaffected (or carrier) child with each pregnancy. After our second loss, there was not much that anyone could have said or done at the time to make us feel hopeful, other than reading stories of couples who had success after experiencing loss. We encourage all couples who are carriers to share their stories as this is what provided us hope, and we hope that our story may provide hope for other couples out there that may be going through a similar experience.

CAF thanks Monisha and Parag for sharing, and we send them our warmest wishes for this special journey! To donate to Monisha and Parag's baby shower fundraiser, visit:http://bit.ly/babygirlvora. If you would like to host your own online fundraiser to support thalassemia medical research, email info@thalassemia.org.

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.

In Memoriam

We regretfully report the loss of Coolely's anemia patient ANTHONY CERVO 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.

For more information email: info@thalassemia.org.

CAF ANNUAL GALA • JUNE 7, 2018

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.

CAF INTERVIEWS DR. TIPPI MACKENZIE ON FIRST EVER CLINICAL TRIAL INVOLVING IN UTERO STEM CELL TRANSPLANTATION FOR ALPHA-THALASSEMIA MAJOR (ATM)

(CONTINUED FROM PAGE 2)

CAF: What are the risks to the fetuses undergoing this trial?

TM: It is important to consider potential risks for both the fetus and the mother. For the fetus, there is a risk from the transplantation procedure which involves a needle stick into the umbilical vein; in some cases, this procedure could result in the fetus needing to be delivered earlier, or not surviving. Since these fetuses already require this needle stick for the blood transfusion, we think adding the stem cell transplant adds only a minimal risk. There is a chance that the stem cells will not survive and the baby will continue to need blood transfusions or another stem cell transplant after birth. There is also a small chance that the transplanted cells could react against the fetus (graft vs host disease), although this has not been seen in the preclinical studies. There is also a chance that the babies may be born extremely early, and require a long period in the hospital, and perhaps not survive. Each family will need to weigh the risks of doing nothing (which almost always results in fetal demise) with a fetal intervention.

CAF: What are the risks to the maternal participant undergoing this trial?

TM: The main risk for the mother is due to the bone marrow harvest that is necessary to obtain the stem cells. The stem cells are harvested from the hip, under anesthesia, and mothers could experience bleeding, infection, and pain after the procedure. There may be risks of anesthesia during pregnancy, which is a consideration for all cases of fetal intervention. They may also become anemic after donating their bone marrow and may need a blood transfusion. Mothers may also have complications after the in utero transfusion/transplantation procedure, which involves placing a needle through the amniotic sac into the fetus' umbilical cord. This could result in an infection or complications leading to preterm labor. Mothers who carry fetuses with severe anemia can have other pregnancy complications and the hope is that the fetal transfusion could protect from those complications.

CAF: What is the eligibility criteria for participants of this trial?

TM: For this trial, we will include fetuses who have anemia because of underlying alpha thalassemia major, at 18-25 weeks of gestation. Because it is important to transplant enough cells, we can only include patients who have adequate numbers of stem cells harvested from the mother's bone marrow. If a fetus has other severe problems that will make it unlikely for them to survive, they would not be offered this therapy. Maternal inclusion criteria are similar to those used in other fetal therapies such as not having signs of preterm labor.

CAF: How many participants will be recruited and what is the length of time they can expect to be involved in the trial? Will all treatments be conducted at UCSF?

TM: We are hoping to recruit 10 participants and will follow them for at least 1 year after the . The initial in utero transplantation will be at UCSF. However, the fetuses in the study will continue to require in utero transfusions every 3 weeks until birth. If a family lives far away, they will be able to go home if there is a provider skilled in performing these in utero transfusions. Follow up for the infant will be either at their home institution or at UCSF.

CAF: Who are the other members of your team working on this clinical trial?

TM: We have a wonderful team of providers skilled in the multiple aspects of care our families will need. For example, Dr. Elliott Vichinsky is a hematologist with extensive experience with this disease. Dr. Juan Gonzalez is a maternal-fetal medicine expert with experience in fetal blood transfusions. Dr. Chris Dvorak is a pediatric hematologist whose team will manage the bone marrow harvest and the processing of those cells for infusion back into the fetus. Kristen Gosnell, our research nurse, will oversee the care of our families through our Fetal Treatment Center. Billie Lianoglou, our genetic counselor, will work with families and referring providers to confirm the diagnosis of alpha thalassemia. Romobia Hutchinson, our program manager, will oversee other aspects of the clinical trial. Finally, there are numerous research faculty and postdoctoral fellows who will be responsible for studying how the stem cells survive in each patient and determine whether we need to make any changes to the transplantation protocol as we begin to obtain results.

CAF: Is there anything else you would like to share with the thalassemia community?

TM: For many years, patients with alpha thalassemia major were not given any options other than pregnancy termination. However, it appears that meaningful survival is possible with fetal transfusions and these are being offered more frequently. We are excited to offer this new therapy of combining the transfusions with a stem cell transplantation since it offers the possibility of a more definitive therapy. We are excited to hear from the community about your opinions about this treatment option.

Additional information for those interested in participating in this clinical trial can be found here: bit.ly/ucsf-clinicaltrial-thalassemia

Flu season is here!

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

Please check with your doctor for further information.

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

CONSIDER A GIFT OF SHARES OF APPRECIATED STOCK

Here's a way to be smarter in the way you support us and perhaps give us even more. And...it's one that has more benefits for you! We're talking about donating appreciated shares of stock.

This may be the best time ever to consider such a gift. Almost all stocks have appreciated in this all-time-high stock market. Hopefully you are among the millions of Americans who have realized such gains and are now in a position to help Cooley's Anemia Foundation with a donation of appreciated stock!

Here are the basic facts about donating appreciated shares of stock:

- You must have owned the shares for more than one year and their value has to have appreciated over the time you owned them.
- If you itemize deductions on your income taxes, you can take a charitable deduction for the fair market value of the stock shares on the day you donate them.
- You do not have to pay capital gains taxes on the increased value of the shares since you bought them. (You would have to pay capital gains taxes if you sold the shares and gave the proceeds to Cooley's Anemia Foundation.)

And here is the information you need to have your broker donate shares of stock to Cooley's Anemia Foundation:

- Ask your broker to donate through Morgan Stanley
- If Sending Electronically:
  - » Morgan Stanley
  - » DTC Number: 0015
  - » FBO Cooley's Anemia Foundation
  - » A/C Number 409-108690-172
- Contact Ali Girardi (203) 625-4845, Ali.Girardi@morganstanley.com

And there you have it! Please remember that all donations to Cooley's Anemia Foundation help make life better for those suffering from thalassemia. On their behalf, we thank you for all you do. It matters.

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.

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RECORD-BREAKING ATTENDANCE AT THE CAF 2017 PATIENT-FAMILY CONFERENCE

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