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.”

Continued on Pg 12
Caf INTERVIEWS DR. TIPPI MACKENZIE ON FIRST EVER CLINICAL TRIAL INVOLVING IN UTERO STEM CELL TRANSPLANTATION FOR ALPHA-THALASSEMA MAJOR (ATM)

The University of California, San Francisco (UCSF) is currently recruiting participants for the first ever clinical trial performing in utero stem cell transplants 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. 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.

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.

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 Pres- ident. 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 1,600 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 $100,000 in four years.
• UFL01, an organization of 7-11 franchise holders in Long Beach, has designated 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 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 that.

With love,
Tony Viola
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 applicants (undergraduate, graduate, 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.

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 toward 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.

CAF 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.

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!

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 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 information on thalassemia adoption, to share the stories and perspectives of adoptive families.

How Do 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 (major) but we did not know my husband was also a carrier. 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 that 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 having will require much effort and you can’t say that the process is easy.

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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 (6), 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?

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.

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. Thrown 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 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!

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, we 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.
For me, advocating for their needs is another challenge. Fighting—more resilient, more caring and empathetic and will one day be stronger—but it is also a HUGE blessing. I believe our boys are stronger, better, more 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.

But it is not going away. This is their life. And on the other side emotional pain of thalassemia. I just wish I could take it away. 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. Cai has also brought me into a deeper relationship with Jesus. It’s such a joy to have a front row seat to his miracles!

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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 understand 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.

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!

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 brings 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!
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 unyielding 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 Davis, Cooley’s Anemia Foundation National President Anthony Viola, Susan Viola, Founder and Chairman of Apotex Dr. Barry Sherman, Honey Sherman, Olga Spira, Honoria Dr. Michael Spira, Pat Kay, Vice Chairman of the Board of Apotex Jack Kay, Rasa Maria Tricta, and Honoria Dr. Fernando Tricta.

Paul Toci, Kathleen Toci, 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 Picerno, and Marianne Picerno.

M:

We regretfully report the loss of Cooley’s anemia patient Anthony Cervo and extend our sympathies to his friends and family.

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.

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 Cooley’s anemia patient Anthony Cervo and extend our sympathies to his friends and family.

Volunteer Spotlight (Cont. from pg. 7)

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 JUNE 6-8, 2018 • ATLANTA, GEORGIA

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

to survive, they would not be offered this therapy. Maternal procedure, which involves placing a needle through the amniotic and may need a blood transfusion. Mothers may also have may also become anemic after donating their bone marrow

TF: 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. Eliott Vichinsky is a hematologist with extensive experience with this disease. Dr. Juan Gonzalez is a maternal-fetal medicine expert with experience in fetal blood transusions. 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 Langlou, 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 transplants and these are being offered more frequently. We are excited to offer this new therapy of combining the transplants 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

CONSIDER A GIFT OF SHARES OF APPRECIATED STOCK

Here’s 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.)

With your help, the cure is within reach!

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