



WINTER 2020

LIFELINE

COOLEY'S ANEMIA FOUNDATION • LEADING THE FIGHT AGAINST THALASSEMIA

CAF'S 8TH ANNUAL PATIENT-FAMILY CONFERENCE INSPIRES HOPE FOR THE NEW DECADE

With news of exciting progress on therapies for thalassemia, the Cooley's Anemia Foundation's 2019 Patient-Family Conference brought hope to over 300 members of the thalassemia community from cities across the United States. The Foundation holds this Conference every year with the goal of providing the latest information on thalassemia care and research, as well as curating a safe space for the thalassemia community to share their experiences and build relationships.

The Conference featured several presentations from experts in the field of thalassemia, including hematologists, nurses, and patient advocates. Dr. Tippi MacKenzie, Professor of Surgery at University of California San Francisco, shared some very exciting results from her ongoing clinical trial on fetal therapy for

alpha thalassemia major. During this trial, stem cells from the mother are transplanted into an unborn fetus with alpha thalassemia major. Dr. MacKenzie reported that the first child ever to receive stem cells before birth was born healthy as a result of this treatment. As the world's first clinical trial on in utero stem cell therapy, the successful results from this study may be able to help doctors treat fetuses with a range of blood disorders. At the end of her presentation, Dr. MacKenzie announced that her team plans to expand this clinical trial to include fetuses with beta thalassemia major in 2020.

Dr. Thomas Coates, Head of Hematology at Children's Hospital Los Angeles, also gave a presentation on the future of thal-

Continued on Pg 11 ➤



CAF EXECUTIVE COMMITTEE MEMBERS

Peter Chieco
President

Janice Cenzoprano
Vice President

Janet Kwiatkowski, MD, MSCE
Medical Advisory Board Chair

Dean Hernan
Treasurer

Maria Hadjidemetriou
Secretary

Cammie Brandofino
Amy Celento
Mary Ann Cervoni-Iaia
Ralph Colasanti
Terri DiFilippo
Robert Ficarra
Frank Somma
Joseph Vigliotti, Esq.

EXECUTIVE DIRECTOR

Craig Butler

LIFELINE EDITOR

Mary Woldegiorgis
National Communications Director

Partial funding for this issue of Lifeline has been provided through a grant from Novartis Pharmaceuticals. Lifeline is a publication of the Cooley's Anemia Foundation • 330 Seventh Ave #200, New York, NY 10001 • www.thalassemia.org



LIFELINE

FROM CAF NATIONAL VOLUNTEER PRESIDENT PETER CHIECO

The new year is a traditional time to be thankful for the blessings one has received. This year is no different, and the Cooley's Anemia Foundation counts among its blessings the following:

We are thankful for all of our patients who continue to live fuller and longer lives than ever before in the history of this disease.

We are eternally grateful for all of our volunteers and donors who provide the necessary support needed to find a cure and better quality of life for all those with thalassemia.

We are in awe of the outstanding medical caregivers who are on the front line of battling this life changing blood disorder.

We are hopeful for the incredible research that we are supporting and encouraging and the potential positive changes yet to come.

We also recognize that for all of the reasons we are thankful, we still are driven to improve all outcomes. The fact remains many patients still succumb to this disease every year, a situation that must change for every patient, anywhere they reside.

One of the ways we can influence this change is to continue to



expand our program of encouraging and funding visits to the major thalassemia treatment centers for comprehensive care examinations. Some of the biggest challenges we face in this regard is lack of awareness of this program and of the many other benefits patients and their families can receive when they engage with the foundation.

If I could wave a wand and create a change, my wish would be that every patient in the U.S. was connected to the Foundation, that all patients visited a thalassemia treatment center that provides expert care, that we create ways to help the very sickest patients outside of our great country, and that our resources will be strong enough to accomplish those objectives. You have our word that we will focus our efforts on making the above wishes a reality, as we work for a universal cure for all.

We have much to be thankful for as we start a new year and decade. But the fact remains, we cannot continue to improve our efforts without the generous support of others. Please keep us in your prayers and connect with us in ways that further our efforts to reach our goals.

Thank you,
Peter

FDA APPROVES REBLOZYL

On November 8, 2019, the Food and Drug Administration (FDA) approved REBLOZYL (luspaterecept-aamt) for the treatment of anemia in adult patients with beta thalassemia who require regular red blood cell transfusions. REBLOZYL (pronounced like "red blood cell") is the first and only FDA-approved erythroid maturation agent, representing a new class of therapy which works by regulating late-stage red blood cell maturation to help patients reduce their RBC transfusion burden.

The approval of REBLOZYL for beta thalassemia was based on results from the pivotal Phase 3 BELIEVE trial, which began in May of 2016. As a result of this clinical trial, 21.4% of patients treated with REBLOZYL achieved a $\geq 33\%$ reduction from baseline in RBC transfusion burden (with a reduction of at least 2 units) during weeks 13–24 after randomization, compared to 4.5% (n=5) in the placebo arm.

"There are very limited options for patients living with anemia due to beta thalassemia who are dependent on long term red blood cell transfusions," said Nadim Ahmed, President, Global Hematology and Oncology for Celgene. "We are pleased to make REBLOZYL available as a new therapy for these patients to help address their anemia, a significant clinical complication of beta thalassemia."

"We're thrilled that Acceleron's first approved medicine is one with the potential to help patients with beta thalassemia, who have been in need of new treatments for this lifelong disease,"

said Habib Dable, President and Chief Executive Officer of Acceleron, "We are enormously grateful to the patients, families and caregivers who participated in and supported our research.

In terms of adverse reactions, hypertension was reported in 10.7% of REBLOZYL-treated patients in the BELIEVE trial. Thromboembolic events, including deep vein thromboses, pulmonary embolus, portal vein thrombosis, and ischemic stroke, were experienced in 3.6% of REBLOZYL treated patients. The most common adverse reactions were headache, bone pain, arthralgia, fatigue, cough, abdominal pain, diarrhea, and dizziness. Permanent discontinuation due to an adverse reaction occurred in 5.4% of patients who received REBLOZYL.

REBLOZYL is now accessible to adult patients in the United States. The Celgene Patient Support Program is ready to help patients and families learn about financial assistance that may be available for REBLOZYL. To enroll in Celgene Patient Support, call 1-800-822-2496 or visit www.celgenepatientsupport.com.

There is currently an ongoing clinical trial of REBLOZYL for the treatment of beta thalassemia in pediatric patients. Results from this pediatric clinical trial are not yet available. CAF looks forward to the results from this study and will share them with the community as soon as they become available.

NAVDEEP SINGH SELECTED FOR CAF-APOPHARMA DISTINGUISHED SCHOLAR AWARD



The Cooley's Anemia Foundation is proud to announce Navdeep Singh as the 2018 CAF-ApoPharma Distinguished Scholar Award recipient. The award is in the amount of \$20,000 and is presented to individuals with a clinically significant form of thalassemia who live in the United States and 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 is delighted to extend our congratulations to Navdeep. Navdeep's goals and aspirations represent values of innovation and a passion for excellence and are what we hoped to recognize in establishing the CAF-ApoPharma Distinguished Scholar award," said ApoPharma President Dr. Michael Spino. "We commend Navdeep for the critical research path he is embarking upon and wish him continued success in this work."

Navdeep's personal experiences as an individual with thalassemia and his exposure to patients with sickle cell anemia as a child inspired his passion for pain management. This passion led him to earn a Bachelor of Science in Nursing from Wayne State University, followed by a Master of Science in Nursing at Madonna University. He now works as a Nurse Practitioner specializing in pain management at the Beaumont Hospital, where he manages patients dealing with cancer pain, post-op pain, acute pain, chronic pain, and pain associated with sickle cell disease.

Navdeep is also currently pursuing a PhD at Wayne State University. He aims to produce research that can be applied in a clinical setting, with a goal of reducing health disparities related to the management of pain. Navdeep has presented at the American Society of Pain meeting, published an article in the journal *Pain Management in Nursing*, and attended the Pain Consortium at the National Institutes of Health. As a result of his exceptional work, Navdeep was chosen as a Robert Wood Johnson Future of Nursing Scholar.

"The Foundation is honored to present the CAF-ApoPharma Distinguished Scholar Award to Navdeep," says Peter Chieco, CAF Volunteer National President. "His pursuit of excellence is fueled by a deep commitment to improving the lives of patients, and we are so proud to support his efforts. CAF thanks ApoPharma for their ongoing support for individuals with thalassemia. We also



thank the Scholarship Committee for their time and dedication in reviewing the submissions."

"I feel very honored and humbled to be receiving this award. This will aid me in pursuing my goal of reducing health disparities, and I am very grateful to CAF and ApoPharma for encouraging thalassemia patients to pursue all they can be." – Navdeep Singh



In addition to his focus on improving health disparities through his research, Navdeep is passionate about making an impact on health outcomes in developing countries. As an undergraduate, he traveled to South Africa and Kenya, where he educated patients with HIV on antiretroviral medications. As a graduate student, Navdeep visited rural areas of Haiti and the Philippines to help run travel clinics. "Doing the volunteer missions reminded me that I wasn't alone in this healthcare struggle," explains Navdeep. "I was inspired seeing that, even though these regions were quite poor, the people were resilient and in good spirits. Having thalassemia limited me from doing a full study abroad because I had to be back home every three to four weeks for a transfusion. However, I did make it a goal of mine to see the world, and I am proud to say I have visited 52 countries. I want to continue traveling, as these trips were life-changing and very humbling."

Navdeep hopes to inspire other individuals with thalassemia to pursue their dreams. "To my fellow thalassemia patients, I want to say that we are a small group, but we are not a defenseless group. Don't let anything stop you from obtaining what you want," says Navdeep. "People are certainly entitled to privacy, but I hope people with thalassemia will be vocal and spread awareness. Get involved with your government so people can see there are faces attached to the 'pre-existing conditions' out there. Be a strong self-advocate in your physician's office and ask, 'When is my MRI, Dexascan, 2D Echo, PFT, referrals to endocrinology, etc.' It's your health, so take charge!"

CAF congratulates Navdeep on receiving the 2018 CAF-ApoPharma Distinguished Scholar Award, and we wish him much success in his future.

CAF PROVIDES GRANT FUNDING TO EIGHT MEDICAL RESEARCHERS

CAF is happy to announce the eight recipients of the 2019-2020 Cooley's Anemia Foundation Medical Research Fellowships and Grants. There are two new clinical research grants, 1 new fellowship and five renewal fellowships. The combined total awarded for these Fellowships is \$312,500.

All applicants were assessed for the quality of scientific content, the academic accomplishments and future promise of the investigator, the quality of the mentor in the case of postdoctoral fellowships, and of special importance, the relevance of the project to the understanding and treatment of thalassemia. Members of the CAF Grant Review Committee carefully reviewed all applications while adhering to the highest standard for scientifically unbiased reviews and provided its recommendations for funding to the CAF Board of Directors, who approved the final selections at its annual Board meeting.

We thank Dr. Janet Kwiatkowski, Chair of the CAF Grant Review Committee and Medical Advisory Board, and to all other members of the Committee for reviewing the applications and offering their recommendations. We look forward to the results of these important investigations.

FIRST YEAR CLINICAL RESEARCH GRANTS



Maa-Ohui Quarmyne, MBChB of Children's Healthcare of Atlanta will be examining patient-family knowledge, perspectives, and expectations of gene therapy and to assess factors that influence decision making about gene therapy. The investigators will conduct up to 50 semi-structured interviews with patients and/or parents and apply

rigorous qualitative analyses to the data. The significance of this study is that no other study has yet examined the patient/family perspectives on risk-benefit ratios and what would constitute an acceptable outcome. As gene therapy rapidly advances and may be clinically available for the treatment of thalassemia within the next few years, the results of this work will help inform the design of clinical trials in gene therapy and methods to counsel patients and their families about this therapy.



Stella T. Chou, MD of Children's Hospital of Philadelphia will examine the blood donor risk factors for red cell alloimmunization in patients with thalassemia. Dr. Chou's previous research involved RBC genotyping of patients with sickle cell disease, revealing a significant variability in the Rh antigens. She then reviewed the genotype profiles of African

American blood donors who phenotyped negative for C, E and K antigens (a desirable profile for a donor) and found that many are not truly negative due to partial antigens. Following these previous findings, Dr. Chou will now be genotyping her local thalassemia population to review the donors that were involved in new alloimmunization episodes via a chart search. If this study shows that increased rates of anti-Rh antibodies in patients with thalassemia are due to exposure to blood donations from African Americans with Rh variants, these findings may support widespread use of genotyping (not just phenotyping) donors to get more accurate matches. This is not currently standard practice because of cost, but as the cost of genotyping decreases, this data would provide strong support for a change in blood donor center practice to lower alloimmunization rates

FIRST YEAR FELLOWSHIP



Xianjiang Lan, PhD of the Children's Hospital of Philadelphia will be working to find ways to increase fetal hemoglobin. Much attention has been focused on modulating transcription repressors BCL11A and LRF/ZBTB7A to increase fetal hemoglobin production. However, finding additional pathways to increase fetal hemoglobin is important to increase

the chance that a patient can be effectively treated. The aim of Dr. Lan's project is to find novel fetal hemoglobin regulators that may be amenable to modulation by small molecules (drugs). Dr. Lan has already identified a new pathway involving the SPOP protein, a repressor of fetal hemoglobin, and plans to identify how SPOP controls HbF levels. If successful, the results of this study could present a new approach to fetal therapy.

in thalassemia patients.

SECOND-YEAR RENEWAL FELLOWSHIPS



Yvette Yien, PhD of the University of Delaware will study the regulation of mitochondrial iron import by FAM210B. Identifying mechanisms by which red cells regulate iron utilization may help better understand the mechanisms of ineffective red cell production. FAM210B is a novel gene whose expression is unregulated in terminally differentiating red

blood cells. In her previous research, Dr. Yien has shown that protein FAM210B is a regulator of mitochondrial iron metabolism that functions to increase heme synthesis and mitochondrial iron transport during terminal erythropoiesis by directing mitochondrial iron towards heme, at the expense of iron-sulphur cluster

formation. This role may be differentiation stage-specific and serve to couple iron metabolism to the specific needs of the cell. Dr. Yien recently published these results in the *Journal of Biological Chemistry*. In her new study, Dr. Yien will further examine the role of FAM210B in metabolic reprogramming and identify the roles of FAM210B in energy utilization within the red blood cell. Since dysregulated iron metabolism is a hallmark of thalassemia, these studies will broaden understanding of thalassemia and guide the design of new treatments.



Divya Vinjamur, PhD of Boston Children's Hospital will examine heterochronic silencing of fetal hemoglobin (HbF) as a therapeutic target for beta thalassemia. In her previous research, Dr. Vinjamur identified that let-7a miRNA directly binds to HBG mRNA, and now will continue working to identify sequences necessary for let-7 binding.

In addition, she plans to validate a set of let-7 target genes and assess their mechanism and level of fetal hemoglobin activation. These studies may lead to the identification of novel therapeutics or contribute to gene therapy approaches to ameliorate thalassemia.



Raman Bahal, PhD of the University of Connecticut will use next generation site-specific genome editing of hematopoietic stem cells for beta thalassemia gene therapy. Gene editing approaches to the treatment of thalassemia carry the risk of off-target breakage in the genome, which can cause cancers or other ill effects.

Dr. Bahal is using an innovative approach designing advanced molecules to wedge open DNA without breaking it, allowing short segments of DNA (PNAs) to enter and fix thalassemia mutations. In his previous research, he synthesized and analyzed GcPNAs targeting a thalassemia mutation and formulated nanoparticles to deliver the GcPNA and donor corrective DNA. Dr. Bahal will now explore the mechanism of PNA-based gene editing and identify novel nano-therapeutics based strategies to deliver the treatment to hematopoietic stem cell cells by IV infusion. If successful, this could lead to a cure for thalassemia that would not require intense chemotherapy.



Chia-Yu Wang, PhD of Massachusetts General Hospital will examine the mechanism of Erythroferrone action to regulate iron loading in beta thalassemia. Iron overload remains a major problem in thalassemia. Although much of the excessive iron in thalassemia major comes from

transfusions, there is also significant contribution from iron in the diet in thalassemia major, and dietary iron is the major contribution to iron overload in thalassemia intermedia. Even when iron overload is present in patients with thalassemia, the increased red cell production in the bone marrow triggers the release of

erythroferrone, which signals the cells in the bowel and elsewhere to absorb and release more iron. Dr. Wang is working to understand the mechanism of action of erythroferrone. Her work to date has shown a possible role of BMP6, BMP2 and ALK3 in hepcidin suppression. Dr. Wang will build on this research during the second year of funding. The results of this work may suggest a new therapeutic approach to the management of iron overload in thalassemia.



Annamaria Aprile, PhD of the Fondazione Centro in San Raffaele, Italy will study how alterations in the bone marrow environment may affect hematopoiesis. Using mice with beta thalassemia, Dr. Aprile has identified alterations in the bone marrow stroma and hematopoietic components of the bone marrow niche which may exhaust hematopoietic stem cells

(HSC). She will expand upon this work to understand the molecular pathways that underlie the beta thalassemia HSC defect. These studies may reveal underlying mechanisms that influence HSC and early progenitor activity in thalassemia, and why this altered marrow leads to ineffective red cell production.

It's Never Too Late to Start Being Physically Active

John didn't become physically active until his mid-30s, when he began to gain weight, develop high blood pressure, and suffer from extreme tiredness. He decided to begin an exercise program to improve his health – and now he's in the best shape of his life!

Becoming fit was such a life-changing experience for John that he now coaches other people to help them achieve their health and fitness goals.



Any amount of physical activity provides health benefits, so do what you can based on your abilities after consulting with your doctor. You don't need to adopt a formal exercise program – you can easily get started simply by walking.

Find activities that get you moving and that you enjoy and make them a regular part of your life!

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

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

PATIENT PROFILE: AMY PIZZULLI

When Amy Pizzulli was born in 1956, her parents were told that the life expectancy for a person born with thalassemia in the United States was 12 to 13 years of age. Throughout her childhood, Amy's mother told her, "We're going to make the most of the time we have together." As Amy moved from childhood to adulthood, her great strength and positive outlook allowed her to face the challenges of living with a chronic illness; she also credits her childhood hematologist (doctor who studies blood disorders) with her relatively good health in childhood and beyond.

Amy pursued a Bachelor's Degree in teaching, and considers her college education as one of the best things that ever happened to her. Just like other college students her age, she enjoyed participating in academic life and didn't want to be treated as different or fragile, despite having thalassemia. She would return home for transfusion treatment, as needed. Her first 3 years of college were a time of great growth and independence.

By her final year of college, the buildup of iron in Amy's heart, a side effect of transfusions, had caused her to go into heart failure. At that point she had great difficulty even turning over in bed. To remove this excess iron, Amy needed chelation therapy. Fortunately, a new chelation treatment (deferoxamine) became available in the United States at this time, so Amy started chelation therapy for the first time at age 21. Between chelation and a more aggressive transfusion regimen, she gradually started breathing and moving better. Although the chelation was difficult to administer, Amy knew it was removing the excess iron from her heart. "I saw the huge difference that chelation made in my life," she said. "Because of that, I never struggled with staying on track with my thalassemia treatment. I knew that chelation had given me a new life."

After graduation, Amy began teaching third grade as an elementary school teacher in New Jersey; she also taught Honors math and science. Being a teacher was extremely satisfying for Amy. It means the world to her now when former students write to thank her for contributing to their success in life. In order to avoid missing work to receive transfusions, Amy began attending the night transfusion clinic at New York Hospital. This turned out to be one of the greatest experiences of her life. For the first time, she was exposed to a large group of fellow patients who served as a support network. Being around them made her feel much less isolated. Hearing other patients' stories helped her realize that if others were able to get through their situations in managing thalassemia treatment, she could as well.

Because of her thalassemia diagnosis, Amy's mother tried to prepare her for the possibility that she might not ever marry; as a result, Amy never felt pressured to find a husband. However, at the evening transfusion clinic, she met a fellow patient named Danny, who was a very positive influence on her; they married within 2 years. "We always felt that, together, we were going to make it," said Amy. Still, she remembers being afraid to buy a house because she wasn't entirely sure what



the future held in store for them – but they bought the house anyway. Danny suggested that they take out a 401(k) plan. Amy recalls thinking, "We're never going to make it to retirement!" Sadly, Danny passed away after 16 years of marriage. It was very hard on Amy to lose Danny, but she got through it with the help of family and friends. Eventually, Amy met Ted. Early in their relationship, she told Ted, very matter-of-factly, that she had thalassemia. He told her that it didn't make a difference to him. They eventually married, and Ted has been extremely supportive as Amy has also managed diabetes and osteoporosis, complications associated with thalassemia.

Amy retired from teaching in 2014 after 35 years of work. "I never expected to make it to retirement. I have lived a great life!" she said.

AMY PIZZULLI'S TIPS FOR OTHERS LIVING WITH THALASSEMIA:

- Surround yourself with people who are positive and who support you. Try not to become fearful about what could happen, as this might cause you to spiral down and ruin all of the good things that you already have.
- Know that as people with thalassemia approach mid-life, new health challenges may arise that have less to do with thalassemia and more with the process of aging. In addition to your hematologist, it is important to have a primary care doctor you can work with to manage aging issues. Stay positive, and don't be overwhelmed by these things. Think of managing all of your health as an investment in your future.
- Stick to your chelation schedule, and be part of a support group that knows what you're going through.
- Set life goals for yourself. And definitely take out a retirement plan!

CDC and CAF thank Amy for sharing her personal story.

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

PATIENTS SHARE A DAY IN THEIR LIVES ON INSTAGRAM

Throughout the past year, CAF has given several individuals with thalassemia 24-hour access to our Instagram account to share glimpses of a day in their lives. These Instagram takeovers have quickly become one of our most popular community outreach initiatives, with hundreds of people from around the world tuning in to see what life is like for people with thalassemia from different backgrounds and walks of life.

The Cammilleri family were the first to do the 24-hour CAF Instagram takeover. Brothers Corbyn and Cai Cammilleri both have thalassemia major, and agreed to share parts of their day, including a trip to the hospital for their routine blood transfusions. Corbyn is in fifth grade and Cai is in fourth grade. The night before their transfusion, everyone in their family, including their sister Callie and their parents, shared a few thoughts on life with thalassemia.

Since the Cammilleri family's CAF Instagram takeover, eight other individuals with thalassemia have shared a day in their lives with the community. Some have been adults, and others have been children. They live in different parts of the country, come from various ethnic backgrounds, and have all types of fascinating careers. But the one thing they have in common is that they have thalassemia.

Many people who follow these Instagram takeovers are individuals who also have thalassemia, and they often leave comments or send messages expressing how amazing it is to see experiences they can so closely identify with. People who do not have thalassemia also follow the Instagram takeovers, and are able to get a better idea of what life is like for people who live with the blood disorder. Our hope is that this series can help the thalassemia community get closer by sharing their experiences, and to inspire more people to donate blood and learn about this blood disorder on a more personal level.

CAF thanks everyone who has participated so far, including Jeff Singh, Daniela Ciriello, Yasmeen Anis, Brooke Way, Noor Altahafee, T.J. Mills, Kamila Saradpon, Shruti Arya, and Corbyn and Cai Cammilleri.

Be sure to follow us on Instagram @cooleysanemia to see the upcoming patient takeovers. Don't have an Instagram account? Download the free Instagram app for iOS, Android, or Windows from your phone's app store and create a free account. Then follow @cooleysanemia so you won't miss any future takeovers.



*Corbyn and Cai
getting transfusion*

CAMPS FOR CHILDREN WITH THALASSEMIA

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

THE PAINTED TURTLE – LAKE HUGHES, CALIFORNIA

July 18-23, 2020: Thalassemia/Hemophilia Camp
www.thepaintedturtle.org

THE DOUBLE H RANCH – LAKE LUZERNE, NY

May 7-12, 2020: Hematology/Oncology Camp
www.doublehbranch.org

CAMP KOREY – MT. VERNON, WASHINGTON

July 26-30, 2020: Hematology/Oncology Camp
campkorey.org

HOLE IN THE WALL GANG CAMP – ASHFORD, CT

June-August, 2020: General Diagnoses Camp
www.holeinthewallgang.org

ROUNDUP RIVER RANCH – GYPSUM, CO

July 8-13, 2020: Blood Disorders & Other Diagnoses
www.roundupriverranch.org

VICTORY JUNCTION – RANDLEMAN, NORTH CAROLINA

June 28-July 1, 2020: Genetic & Neurological Disorders
www.victoryjunction.org

CAMP CAREFREE – STOKESDALE, NC

July 26-August 1, 2020: Hemophilia/Blood Disorders
www.campcarefree.org

NORTH STAR REACH – PINCKNEY, MICHIGAN

July 21-26, 2020: Sickle Cell/Hematology Camp
www.northstarreach.org

CAMP HOLIDAY TRAILS – CHARLOTTESVILLE, VA

June-July, 2019 – Mixed Diagnoses
www.campholidaytrails.org

CAMP BOGGY CREEK – EUSTIS, FLORIDA

June-August, 2020 – Various Camps
www.boggycreek.org

FLYING HORSE FARMS – MT. GILEAD, OHIO

July 17-22 – Hematology/Oncology/Rare Diseases
www.flyinghorsefarms.org

GLOBAL COMMUNITY CELEBRATES FOURTH ANNUAL THRIVING WITH THAL WEEK

Since 2016, CAF has celebrated our annual Thriving with Thal during the third week of June in celebration of the tenacity of thalassemia patients around the world. The aim of Thriving with Thal week is to illustrate that despite the many challenges that come with thalassemia, patients persevere and find ways to thrive every day. Folks from different corners of the world and all walks of life share their unique stories and perspectives on how they are thriving with thalassemia. Check out more of these amazing stories by searching the hashtag #ThrivingWithThal on Facebook, Instagram and Twitter. Thank you to everyone who participated for sharing your incredible stories!



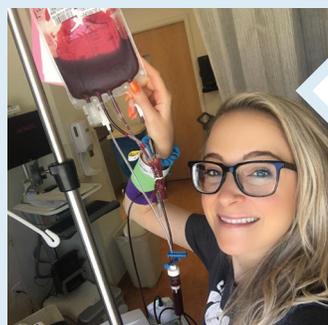
"I am forever grateful for our blood donors who allow me to Thrive with Thal while taking care of my baby girl." -Alyssa

"My daughter Scarlett is 8 years old and has beta thalassemia major. Scarlett receives blood transfusions every 2 weeks and chelates daily. Although she gets tired very easily and hates being tied down to her transfusion pole, she makes the best out of her day at clinic by drawing, coloring, singing and dancing. She is active year-round either cheering or playing softball, which is challenging for her since she can't run around for long periods of time. She works very hard keeping up with the other kids and never ceases to surprise me with her strength and willingness to try her hardest. She doesn't let having thalassemia discourage her from doing things she wants to do and she has the most positive look at life. Certainly can learn a lot from this little girl's outlook on life!" -Vincenza

"My mission in life is not merely to survive, but to thrive; and to do so with some passion, some compassion, some humor, and some style... #Thalassemia is a big part of me but it's not the only part. I also like Chocolate, Gaming, the color purple, Chick Fil A, Sleeping, Snow Tha Product...oh, and cake. Yes, cake makes me a happy, inspirational vampire who's #thrivingwiththal." -Ryan



"It's Thriving With Thalassemia week! I've been reflecting on my condition and what it means to have Thalassemia, and I've realized the following: 1) having to get transfused every 6 weeks sucks 2) spending all day in the hospital sucks even worse...but 3) having Thalassemia has made me the strong, independent fighter I am today 4) has made me thankful for every moment I am alive 5) and has allowed me to meet incredible people who are also struggling with this disease. As much as this condition is exhausting and frustrating, I am and will continue to keep thriving and hoping for a cure. " -Brooke



"My name is Fathia and I live in Palestine. I'm 28 years old and I have both thalassemia major and diabetes. I am an electrical engineer working for the Palestinian Ministry of Health. To all thalassemia patients, I want to say that "Thalassemia means power." Why are we strong people? Because we have a reason to be weak, but we choose strength and refuse the weakness. Thalassemia is a companion we don't choose, but we do choose to be people who thrive with it, and live full of hope and energy and beautiful dreams." -Fathia

"I am Dr. Ravindra Kumar and I have beta thalassemia major. I live in Jabalpur, India and work as a scientist for the Indian Council of Medical Research (ICMR-NIRTH). Strong will power, adherence to treatment and helping hands of blood donors, physicians and nurses help me in thriving with thalassemia." -Ravindra





“My name is Isha. I am 24 years old and have beta thalassemia major. I was born in India and was raised in an orphanage for the first five years of my life. At age five, I was adopted and brought to the United States. This is when I began receiving proper treatment for my thalassemia. At that time, the doctors said that I would not live past 10 years old. However, now that I have been receiving proper care for many years, my health is much better than ever before and I am almost finished with my master’s degree in public administration. I plan to work with health-related nonprofits so that I can give back.

To other people with thalassemia, I want to remind you to keep fighting and never give up because you have a purpose on this earth. That’s what has kept me going. I know there’s a special reason I was born with this illness and I can only understand that reason if I keep going.” -Isha



“Hey! I’m Janaína, I am 30 years old and live in Rio de Janeiro, Brazil. I have thalassemia intermedia and began receiving medical treatment when I was diagnosed at 11 years old. Before that, I remember being in a constant state of pain and exhaustion. Now that I receive blood transfusions every 3 weeks, I’m a happy veterinarian, and also taking courses towards a Master of Science in Public Health.

I think the most difficult thing about living with thalassemia is the struggle with self-esteem. It’s not easy to go through all we do. Sometimes you become really destructive. But I’m really thankful to my family and friends for always being so supportive. I’m also grateful to have access to a professional therapist who helps me deal with my struggles. We all have struggles. But we all can pass through them, too.”-Janaína

“My name is Fadel and I am 25 years old. I live in Jakarta, Indonesia with my mother and my sister. I graduated from college in 2015 with a degree in Informatics Technology (IT). I have been a Thalassemia Warrior since I was diagnosed with thalassemia major at 8 months old.

Since childhood, I have always dreamed of raising awareness about thalassemia in my country and throughout the world. In 2016, I joined a thalassemia community in Indonesia called Thalassemia Movement. This community consists of Thalassemia Warriors, volunteers and people who care about thalassemia. We always campaign to raise awareness about thalassemia to encourage genetic testing so that the number of sufferers in Indonesia does not increase. Our motto is “Thalassemia Movement: We Care, We Share! #StopThalassemia.”

I aspire to be an example for my thalassemic friends in Indonesia. I want to show that thalassemia does not have to prevent us from progress and success. Since 2016, I’ve served as Digital Campaigner and Social Media Content Creator for Indonesia’s Vice-Presidential candidate Sandiaga Uno. Sandiaga hired me for the job without discriminating against me because of my thalassemia. According to him, thalassemia is not a barrier to progress and getting a decent job because people with thalassemia can compete right alongside everyone else. He says that I have proven that I can fight, survive and even surpass others. Having someone I admire and respect give me this type of encouragement means so much to me. I am inspired to do all I can to encourage others with thalassemia to also fight for their dreams.

Something about living with thalassemia that is personally difficult for me is the perception people have when they notice a visible physical difference between me and them. But finally I found a way to improve my confidence about this. I remind myself to just be myself and to not focus on what other people might think or say about me or the way I look. To all Thalassemia Warriors in Indonesia and throughout the world, be yourself, keep on fighting, keep up the spirit and “Enjoy Every Moment.” -Fadel



CAF CELEBRATES TEN YEARS OF CARE WALK IN 2020



This May, thousands of thalassemia advocates in cities all over the United States will gather to celebrate the 10th annual Cooley's Anemia Foundation Care Walk. Thanks to the leadership of dozens of volunteer Care Walk captains, and the support of thousands of walkers, hundreds of donors, and several special corporate sponsors, the Cooley's Anemia Foundation has made incredible strides over the past decade.

Since 2011, Care Walk has:

- Raised over \$2.2 million
- Provided funding that helps to make our invaluable Patient-Family Conferences possible for the thalassemia community
- Helped provide funding for 81 thalassemia research grants
- Helped provide Patient Incentive Awards to 56 students with thalassemia to support their pursuit of higher education

This has all been possible because of incredible advocates who care enough to rally their communities to show individuals with thalassemia just how much they are loved and to ensure them the best future possible. We want to thank each and every one of you for making Care Walk what it has become—a cornerstone for so many family and community memories. The children, especially, are growing up knowing that they have an entire village of people who love them so much and will keep fighting for them.

We want to have a bigger impact than ever this year, and are setting our national Care Walk goal at \$300,000 for 2020.

With your help, we can do it!

CARE WALK: MAY 3, 2020

REGISTRATION IS NOW OPEN

Visit bit.ly/carewalk2020 to register today.

JOIN OUR FACEBOOK GROUP FOR CAPTAINS

Exchange Care Walk ideas and advice, and get to know your fellow Captains by joining our Facebook group for Care Walk Captains. May will be here before we know it, so let's all join forces and share planning strategies in the months leading to the big day! We especially encourage new volunteers to join to get valuable tips from the vets. [Join at bit.ly/CaptainsGroupFB](https://www.facebook.com/CaptainsGroupFB).

Partner with Your Healthcare Team to Take Charge of Your Health

When Tracy transitioned to a new medical center, her new providers had limited experience with thalassemia.



This ended up being a very rewarding experience for her as it forced her to speak up for herself to make sure that her medical needs were being met.

Tracy and her healthcare team now work together as partners – and she's getting the best care of her life!

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



In Memoriam

We regretfully report the loss of patients

PUNEET DHIMAN & MICHAEL DIFILIPPO

and extend our sympathies their friends and families.

PUSH-UPS TO FIGHT THALASSEMIA CHALLENGE TAKES OFF



On February 21, 2020, supporters around the world will be doing push-ups in support of individuals with thalassemia as part of our second annual Push-Ups to Fight Thalassemia Challenge. CAF launched this initiative last year to provide the community with an opportunity to improve their physical fitness while also raising awareness and funds to help individuals with thalassemia.

To participate in the Challenge, supporters create a fundraising page and commit to completing a set of push-ups on the Challenge day, which will be February 21, 2020 this year. Each participant decides for themselves how many push-ups to set as their personal goal. Then they practice doing push-ups in the weeks and days leading up to the big day, all while recruiting friends and family to support them by donating to their fundraising page. To join the Challenge, visit fundraising.thalassemia.org/pushupsthal.

Last year, over 100 people participated in the Challenge, raising more than \$33,000 to fund medical research and patient support programs. Participants posted videos of themselves completing their push-ups on social media with the campaign's hashtag, #PushUpsThal. As more participants uploaded their videos, the campaign started to take off. People from all over the world started asking what this #PushUpsThal campaign was all about, and once they learned about thalassemia and how CAF is trying to raise awareness about the blood disorder and funds for research, amazing things started to happen.

NY Yankees veteran Mark Teixeira sparked an incredible amount of interest amongst his fans when he shared a video of himself completing a set of push-ups for the Push-Ups to Fight Thal-

assemia Challenge in his weight room, complete with a Yankees logo carpet and his framed jerseys on the wall behind him. Some of Mark's fans who saw his video were players on the Mount Saint Joseph High School Gaels Baseball Team, and they decided to take on the Challenge as a team.

The next evening during their workout, the Gaels video taped themselves completing their push-ups as a team and shared it on social media. This ripple effect continued, and people around the country shared their videos to show their support—we even had participants in Italy and Iran!

We want to thank everyone who participated last year for your support. We look forward to seeing your videos this year!

JOIN THE 2020 "PUSH-UPS TO FIGHT THALASSEMIA" CHALLENGE

Complete the challenge between January 1 and February 21, and be sure to share your pictures on social media using #PushUpsThal.

REGISTER TODAY AT [BIT.LY/PUSHUPSTHAL](https://bit.ly/pushupsthal)

[CONTINUED] CAF'S 8TH ANNUAL PATIENT-FAMILY CONFERENCE INSPIRES HOPE FOR THE NEW DECADE

assemia treatment. Dr. Coates shared that the future is bright because curative treatments are already possible, and the approaches and availability will improve over the next decade. He highlighted that decisions regarding overall treatment, especially transplant approaches, require collaboration between patients and providers with broad understanding of all aspects of thalassemia. To ensure such collaboration, Dr. Coates emphasized the need for comprehensive care for every individual with thalassemia. In the United States, there are several comprehensive thalassemia treatment centers and CAF offers a travel stipend to help patients cover the costs of travel. Dr. Coates closed his presentation by encouraging patients to receive a comprehensive care evaluation at one of these centers every year and follow all recommendations for a healthy lifestyle, not only to achieve the best possible health, but also

to prepare their bodies for curative therapy options.

Other speakers covered a variety of topics, including iron management, overcoming issues with insurance, and adopting children with thalassemia. The Conference also provided attendees with the opportunity to participate in group breakout sessions focused on parents, children, teens, adult patients, patients who are also parents, and friends, spouses and partners. Of course, it wouldn't be the CAF Patient-Family Conference without fun activities for families to socialize and get to know each other better informally. As usual, the Saturday night dinner dance was a huge hit among young and old alike, with hours of great food, music and dancing.

CAF thanks all who attended last year, and we look forward to seeing you in July for the next Conference!

CAF'S ANNUAL GALA SHINES A GLOBAL SPOTLIGHT ON THALASSEMIA

Cooley's Anemia Foundation hosted our annual Gala on June 6, 2019 at the Lighthouse at Chelsea Piers. Over 300 supporters joined us for the event, helping raise over \$375,000.

For more than six decades, U.S. families have depended on the Cooley's Anemia Foundation to help them meet the daily challenges of living with Cooley's anemia, and the need is still strong and urgent. Children born with this genetic blood disorder require lifelong blood transfusions as often as every two weeks and must undergo daily drug treatments to remove toxic iron. This delicate balancing act is not without substantial long-term risk even in developed countries. In emerging countries, like China, the prognosis is dire.

But the Foundation's focused efforts are bearing fruit and our community is entering a new era of hope. In addition to new potential treatments, gene therapy has taken tremendous and exciting leaps in recent years, and may be curative. It's a new frontier, and the Foundation is ready to make the future brighter, healthier and fuller for all thalassemia patients around the world. Our shared goal is "Every patient, anywhere, deserves the opportunity to live a normal life span."

To reflect this goal, the theme of CAF's 2019 Annual Gala was "A Global Spotlight on Thalassemia". Our Gala honorees reflect this new reality perfectly. Robert Ficarra, our 2019 Humanitarian of the Year, is currently and has been an active board member of the Cooley's Anemia Foundation for forty-four years, serving as President of the Foundation from 1978 to 1990. During Bob's presidency of the Cooley's Anemia Foundation, the Foundation and five other national thalassemia societies met in Milan, Italy in 1986 to establish the Thalassemia International Foundation (TIF). Bob later served as president of TIF for 8 years. TIF now has representatives from 69 countries. Bob's father, Frank Ficarra, founded the Cooley's Anemia Foundation in 1954, demonstrating what patients and family members can accomplish when they work together. As his son, Bob is proud to carry on that tradition.

Maria Hadimetriou, who received our 2019 Patient Recognition Award at the Gala, was born with thalassemia. Since diagnosis at 2 1/2 years of age, Maria has received over 1,700 pints of blood. She is a mother to her beautiful daughter Julia who just turned 11 years old. She is an experienced real estate sales agent and a freelance writer. She has produced and written a short documentary Thalassemia: Life Without Boundaries and her article, "Thalassemia: Yesterday, Today, Tomorrow," was published in the American Journal of Hematology. Additionally, Maria has been on the Board of Directors of the Cooley's Anemia Foundation

"to everyone here tonight for our cause to find a cure... for now, your love is a cure. That is for sure." – Maria Hadimetriou

since 1998 (currently serving as Secretary of the Board). She has spoken at medical conferences and pharmaceutical firms around the world. For fun, Maria travels and has been to over 20 countries in Europe and the Middle East, and has lived in Cyprus and Italy. During her acceptance speech, Maria thanked all of the Gala attendees during her acceptance speech, saying "to everyone here tonight for our cause to find a cure... for now, your love is a cure. That is for sure."

Debi Mazar an acclaimed actress who made her feature film debut in Martin Scorsese's Goodfellas, served as host for the Gala. A native New Yorker, she is best known for her outstanding portrayals of edgy, sharp-tongued characters on film, television and stage. Her extensive onscreen work includes films with legendary directors Woody Allen, Michael Mann, Oliver Stone and Joel Schumacher. Debi was gracious enough to take photos with everyone who asked for one.

We want to thank everyone who attended for making the 2019 CAF Annual Gala such a success—not only for your generous donations, but also for your ongoing show of genuine compassion and love. To quote Maria's acceptance speech, "your love is a cure."



Gala Chairman, Frank Fusaro, opening the evening's proceedings.



Chris Ficarra (left) and Glenn Ficarra (right) present Humanitarian of the Year award to their father, Bob Ficarra (center)



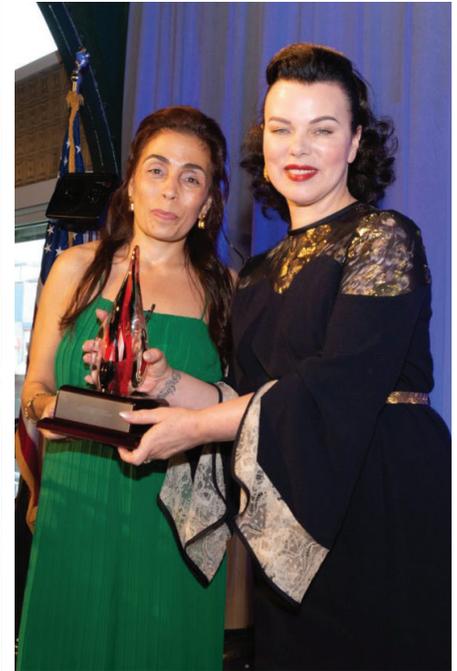
Officers of New York State OSDIA with Tony Viola (far left), Immediate Past Volunteer President of CAF, and Peter Chieco (far right), Volunteer President of CAF



CAF Volunteer National President, Peter Chieco, delivering his welcoming remarks.



A view from the podium of some of the more than 300 guests.



Debi Mazar presenting the Patient Recognition Award to Maria Hadjidemetriou

COOLEY'S ANEMIA FOUNDATION ENDOWMENT

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

WHERE DOES FUNDING COME FROM?

The Endowment is funded through the generosity of donors. Anyone wishing to donate to the Endowment may do so with a gift of \$25,000 or more. There are several different kinds of donations that can be made to the Endowment:

- Pledged Cash Gift – a specified amount to be paid in instalments over a set period
- Gift of Appreciated Stock – with a value at or above a pledged amount
- IRA Charitable Distribution – allows donors age 70 ½ and up to give up to \$100,000 through a qualified charitable distribution
- Gift of Property – real estate or other property with an appraised value at or above a specified pledged amount
- Bequest – naming CAF as a recipient of a set amount in a will
- Insurance Policy – naming CAF as the beneficiary of an insurance policy

- Financial Instruments – various financial instruments (charitable gift annuity, charitable remainder trust, charitable lead trust, donor advised fund, private foundation) set-up with a professional financial advisor that names CAF as the recipient of a set amount or more, either at a set time or upon the death of the donor

It's important to note that many of these options feature some form of delayed fulfillment, which can make them more attractive to a donor. A person may be much more willing to donate \$25,000 if they can do so over a 3-year period, for example. And designating CAF to receive monies from a life insurance policy, will, or retirement plan is one way that a donor can continue to make regular donations to CAF while still contributing to the Endowment.

WHAT WILL THE MONEY BE USED FOR?

All monies will be used to fund the Endowment, and the proceeds from the Endowment will be used to fund Cooley's Anemia Foundation programs.

HOW ARE CONTRIBUTORS RECOGNIZED?

All contributions to the Endowment will be acknowledged in perpetuity by naming the contribution after the donor. For example: The Smith Family Fund.

HOW CAN I DONATE TO THE CAF ENDOWMENT?

To learn more about the Endowment and to donate, email Craig Butler at cbutler@thalassemia.org.

LEGACY SOCIETY REGISTRATION FORM

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

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

- Last Will and Testament
- A Codicil in a Will
- Individual Retirement Account Charitable Rollover
- Charitable Gift Annuity
- Gift of Life Insurance
- Gift of Real Estate
- Retirement Plan Beneficiary Designation
- Charitable Remainder Trust
- Charitable Lead Trust
- Transfer on Death (TOD) on Account
- Contribution to Endowment
- Other _____

We/I have made our/my designation to **Cooley’s Anemia Foundation, Tax ID # 11-1971539, 330 Seventh Avenue, Suite 200, New York, NY 10001.**

- We/I would like for my/our planned gift to be:
 - Unrestricted – for maximum flexibility to meet future needs.
 - Restricted – please share your intentions with us so we can ensure that they are consistent with the Gift Acceptance Policy of Cooley’s Anemia Foundation. Contact us so we can help you with this important decision.
- Please enroll us/me in the Cooley’s Anemia Foundation Legacy Society.
 - You may publish our/my name(s) as (a) society member(s).
 - Please consider this to be an anonymous gift.
- Please send more information about including Cooley’s Anemia Foundation in my/our estate plans.

NAME(S) _____

ADDRESS _____

CITY _____ STATE _____ ZIP _____

PHONE _____ E-MAIL _____

SIGNATURE _____ DATE _____

SIGNATURE _____ DATE _____

Please return to:
Cooley’s Anemia Foundation, 330 Seventh Avenue, Suite 200, New York, NY 10001

Telephone: (212) 279-8090, ext.201
Email: info@thalassemia.org

CAF PARTNERS WITH FREEWILL TO PROVIDE A NO-COST LEGAL WILL

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

Here's how it works:

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

Remember that there are no strings attached to this service.

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

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

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

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

WITH YOUR HELP, THE CURE IS WITHIN REACH!



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

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

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

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.

ZYNTEGLO GENE THERAPY LAUNCHES IN GERMANY FOR TREATMENT OF BETA THALASSEMIA


(autologous CD34⁺ cells
encoding β^{A-T87Q} -globin gene)

On January 13, 2020, bluebird bio announced the launch in Germany of ZYNTEGLO (autologous CD34⁺ cells encoding β^{A-T87Q} -globin gene), the first gene therapy approved in

Europe for the treatment of transfusion-dependent beta thalassemia. ZYNTEGLO is a one-time gene therapy for patients 12 and older with transfusion-dependent beta-thalassemia who do not have a B0/B0 genotype, for whom hematopoietic stem cell transplantation is appropriate but for whom an HLA-matched donor is not available. This is the first time that ZYNTEGLO is commercially available anywhere.

ZYNTEGLO addresses the underlying genetic cause of transfusion-dependent beta thalassemia, and offers patients the potential to become transfusion independent. bluebird bio is working to create qualified treatment centers that will administer ZYNTEGLO, and has established a collaboration with University Hospital of Heidelberg as the first qualified treatment center in Germany.

“For patients with TDT, lifelong chronic blood transfusions are required in order to survive. We are thrilled to announce that ZYNTEGLO will now be available for patients in the EU living with this severe disease,” says Alison Finger, bluebird bio’s chief commercial officer. “In addition to confirming manufacturing readiness of our partner, apceth Biopharma GmbH, bluebird has also submitted a dossier to the Joint Federal Committee (G-BA) in Germany for drug benefit assessment. We would like to thank our collaborators for their commitment in helping us transform the healthcare system by accepting innovative payment models, and we look forward to treating our first commercial patient soon.”

As for the United States, the Food and Drug Administration (FDA) granted LentiGlobin for β -thalassemia (the US name for ZYNTEGLO) Orphan Drug status and Breakthrough Therapy designation for the treatment of beta transfusion-dependent beta thalassemia. LentiGlobin for β -thalassemia is not currently approved in the United States.

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

DISCLAIMER: The information in this publication is for educational purposes only and is not intended to substitute for informed medical advice. You should not use this information to diagnose or treat a health problem or disease without consulting a qualified health care provider. The Cooley's Anemia Foundation strongly encourages you to consult your health care provider with any questions or concerns you may have regarding your condition.

