SICKLE CELL DISEASE
Accessing The New Frontier

Webinar 20 May 2020 at 7:30 am PST/10:30 am EST/ 3:30 pm GMT/ 4:30 pm Central European

Sickle Cell Disease can be considered a global disease, in that it affects patients in many countries, including the US, countries in Europe, Africa, the Caribbean, the Middle East, and India. It affects children, as well as adults.

Have you been diagnosed with Sickle Cell Disease?
Does your child have Sickle Cell Disease?

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There are 7000 rare diseases, impacting about 350 million patients worldwide. Most of these patients have no, or limited access to effective treatments. Putting Rare Diseases Patients First!® seeks to provide actionable information about rare disease new medicine development to patients with rare diseases and the parents of children with rare diseases. The organization decided that the focus in 2020 would be Sickle Cell Anemia, and in particular raising awareness about curative approaches that we believe will be the mainstay of treatment in the future for more and more patients with Sickle Cell Anemia.

Welcome

We welcome you to take part in the Webinar entitled Sickle Cell Anemia – The New Frontier. This is the tenth Webinar in a special series of Webinars. These Webinars allow patients to hear directly from experts and to ask questions. They are open to patients, parents, advocates, professionals, but especially patients and parents. The Webinar will be streamed by FaceBook Live on the PRDPF! FaceBook page.

Goals of the Webinar

Sickle Cell Anemia is caused by a single genetic mutation. During the Webinar we will not spend a lot of time on the symptomatic treatment of Sickle Cell Anemia. Why? Firstly, we have a limited amount of time. Secondly, many patients are experts on their Sickle Cell disease, and are very familiar with the symptomatic treatments that are available. With the limited time available for the Webinar, we want to provide information on the curative approaches. Many patients are unaware of these because most patients and parents would not hear about these potentially curative approaches unless their disease is severe or poorly managed.

Building Trust

Amongst the community of patients with Sickle Cell Disease, there is a level of distrust about participation in clinical trials. We are very grateful to the experts who have cleared their calendars to take part in this Webinar. We understand that the only way to address distrust is to build trust. To this end, we will seek to address questions from patients and parents in an open and transparent way.

Sponsor

The Webinar, Sickle Cell Disease– The New Frontier, is sponsored by Sickle Cell Foundation Nigeria.

Sickle Cell Foundation Nigeria - Advancing Sickle Cell Prevention, Treatment, Care, Cure, and Research

Dr. Annette Akinsete

Consultant Public Health Physician,
National Director and CEO of Sickle Cell Foundation, Nigeria

Annette Akinsete is a woman of many giftings - a Consultant Public Health physician, a teacher and a broadcaster. She is currently the National Director and CEO of Sickle Cell Foundation Nigeria.

Dr. Akinsete was Director (Public Health Department) in the Federal Ministry of Health for many years and has represented the Federal Government of Nigeria at several conferences worldwide. As a Public Health Physician, she has worked in every level of government in Nigeria – local, State and Federal. At global level, she has contributed to World Health Organisation (WHO) publications on Non-Communicable Diseases (NCDs), served as WHO Fellow in Geneva and as UN Cares Facilitator at the United Nations HQ in New York. A member of the Board of University of Ibadan Research Foundation, Dr. Akinsete continues to support the formation of young girls as a Brownie Owl.

AGENDA

Welcome

Dr. Lorna Speid, Founder and Chair of Putting Rare Diseases Patients First!®
Welcome of all dignitaries, Sponsors and Supporting Patient Foundations

Few words from Dr. Annette Akinsete - Sickle Cell Foundation Nigeria (Sponsor)

Few words from Professor Weiss, St. Jude Children's Hospital, Memphis, TN, USA

Few words from Mrs. Agnes Nsofwa, RN, MsN, BBA , Founder and Executive Director, Australian Sickle Cell Advocacy, Inc.

Keynote Presentation

Dr. Elizabeth Klings, Boston University Hospital

Presentation

Kemi Williams, 54Gene – The place of genetics in Sickle Cell Disease

Interview

Professor Bosede Bukola Afolabi, Lagos University, Idi-Araba, Lagos, Nigeria

Presentation

Stem Cell Transplantation for Sickle Cell Disease – Dr. Gary John Schiller – David Geffen School of Medicine at UCLA, California, USA

Presentation

Dr. Akshay Sharma, St. Jude Children's Hospital, Memphis, TN, USA
The place of Stem Cell Transplantation, Gene Therapy and CRISPR

Patient Interview

Mrs. Scherika Perry and Rhiannon Perry – *Living in the New Frontier*
Hear from a patient who has experienced a cure of Sickle Cell Disease and Lupus

Closing comments from Patient Organizations

Close - Dr. Lorna Speid/ John Wood, Board Secretary

Bios for the Chair and Speakers

Lorna Speid, Ph.D., B.Pharm.(Hons.), M.R.Pharm.S.
Founder and Chair
Putting Rare Diseases Patients First!®

Lorna is a new medicine developer, with experience with many different therapeutic areas. She has always been interested in diseases that are unmet medical needs. Dr. Speid has experience developing new treatments for many therapeutic areas, including oncology (hematological and solid tumors), diabetes, obesity, anti-infectives (anti-bacterial and anti-viral), pulmonary (asthma, COPD), influenza, women's health (hormone replacement therapy), bone (Paget's disease and osteoporosis), lupus, Rheumatoid arthritis, transplantation, autoimmune diseases, Malaria, and CNS (psychiatry, Alzheimer's Disease). She has a special practice in rare diseases, and another in neglected diseases. In all of these areas, she develops regulatory strategies, as well as operational approaches that can be used to secure regulatory approvals around the world. Lorna has worked with all treatment modalities, including small molecules, large molecules, gene therapy, combination products (drug and device), companion diagnostic approaches, cellular products, and Biosimilars. She has experience working with oral, injectable and topical medications.

Lorna has worked for large pharma as well as small biotech companies, including Sanofi Winthrop in the UK, Ciba Geigy and Novartis in Switzerland (at Headquarters). Small companies that she has worked for include GeneMedicine/Valentis, Inc. (Director of Regulatory Affairs), NewBiotics (Vice President Regulatory Affairs and Project Management including QA oversight), and Avera, Inc. (Vice President of Regulatory Affairs). Dr. Speid was an officer at the last two companies. She founded and incorporated Speid & Associates in 2004. Since that time, she has been able to use her expertise to make a difference for many other companies and organizations.

Dr. Speid is the author of *Clinical Trials: What Patients and Healthy Volunteers Need to Know*, which was written for patients and healthy volunteers. This book was published by Oxford University Press in 2010. Lorna started Putting Rare Diseases Patients First!® in 2014 to give back, and allow others with expertise to give back to patients with rare diseases. The goal has always been to provide information about new medicine development for rare diseases, directly to rare disease patients and parents. The organization uses social media, Webinars, Blogs, and other technologies.

Elizabeth S. Klings, MD
Associate Professor in the Department of Medicine
Director for the Center for Excellence in Sickle Cell Disease
Director of the Pulmonary Hypertension
Boston University Medical Center

Dr. Klings is an Associate Professor in the Department of Medicine. She is the Director of the Center for Excellence in Sickle Cell Disease and the Director of the Pulmonary Hypertension at BUSM/Boston Medical Center. She has an outpatient clinical practice focused on patients with pulmonary hypertension and those with pulmonary complications of sickle cell disease. She attends in the Medical Intensive Care Unit and on the Pulmonary Hypertension Consultation Service at Boston Medical Center. As Director of the largest sickle cell center in New England, Dr. Klings has created numerous new clinical programs for her patients and has expanded the clinical trials program.

Dr. Klings' research since fellowship has focused on pathogenesis of pulmonary vascular complications of sickle cell disease. She is an NIH-funded Principal Investigator .in the Pulmonary Center collaborating with numerous other investigators across the medical campus. She has phenotypically screened over 200 sickle cell patients at Boston Medical Center for the presence of pulmonary hypertension and currently investigating the roles of disease modulators including sleep-disordered breathing and venous thromboembolism in the endothelial and vascular dysfunction these patients experience. Known internationally as a leader in the care and treatment of patients with sickle cell disease, she was first author of the American Thoracic Society sponsored clinical guidelines for Diagnosis and Treatment of Pulmonary Hypertension in Sickle Cell Disease. She recently chaired an ATS sponsored workshop to define clinical and research priorities in sickle cell lung disease and serves on the NHLBI Sickle Cell Disease Advisory Committee

Kemi Williams, MBA
VP Clinical and Regulatory Affairs
54Gene

Kemi Williams is a strategic and results-driven biotechnology leader with over 19 years combined experience in the pharmaceutical, medical device and diagnostics industries.

Her focus at 54gene is on establishing clinical and regulatory services with enabling infrastructure for the successful conduct of clinical trials in Africa and beyond. She brings years of driving clinical excellence, regulatory competence, and operational efficiency to 54gene as the company of choice for end-to-end clinical development services.

Prior to joining 54gene, Kemi held positions of increasing responsibility at major biotechnology companies, such as Roche, Abbott and Medtronic. Most recently, she was the Head of Clinical Affairs for Siemens Healthineers US molecular business, where she led the development of strategies, structures, and processes for delivering global clinical studies. Some of her accomplishments include active leadership of clinical trials that supported the successful launches of multiple abdominal aortic aneurysm stents, a mitral regurgitation clip for heart failure patients, and multiple in-vitro diagnostic assays and instruments for oncology, infectious diseases and women's health.

Kemi is passionate about advancing organizational strategies and is driven by a vision to deliver impactful healthcare solutions, through regulatory affairs oversight, clinical evidence generation, and scientific marketing. She holds a B.Sc, Computer Science from Loyola Marymount University, Los Angeles and an MBA from California State University, Monterey Bay.

Bosede Bukola Afolabi, MBChB, DM (NOTTS), FRCOG
Healthcare Provider
Professor, Lagos University Teaching Hospital
Idi-Araba, Lagos, Nigeria

Bosede Afolabi is a Professor of Obstetrics & Gynaecology, trained in Nigeria and the United Kingdom. She is a maternal medicine expert and her doctoral thesis was on cardiovascular aspects of pregnancy in sickle cell disease. She and her team have discovered the reversal of certain hormones in pregnant women with sickle cell disease, leading to the recent award of a N38.5m grant (approx. 100,000 USD) for a clinical trial as a follow up of their work. She is also co-Investigator on several grants including a $3.2million Fogarty/NIH grant (no 5D43TW010134-03) grant for improving research and education in junior faculty several research , has published 55 peer-reviewed papers and was featured on CNN African voices for her work in sickle cell pregnancy and maternal health

(http://edition.cnn.com/video/#/video/international/2013/02/01/bosede-afolabi-sickle-cell- anemia-a.cnn)

Bosede is the current Head of Department of Obstetrics and Gynaecology at the College of Medicine, University of Lagos, one of the oldest and most renowned medical schools in Nigeria. She is a fellow of the Royal College of Obstetricians and Gyanecologists, UK and of the Nigerian postgraduate colleges – West African College of Surgeons and the National Postgraduate Medical College of Nigeria. She also has a certificate in Epidemiology and Biostatistics from Harvard School of Public Health, Boston, USA. She is a mentor to several clinical academicians, four of whom have received seed grants of between 10,000 and 12,000 USD each, under her supervision, to carry out various small studies in Obstetrics and Gynaecology.

Gary John Schiller, M.D.
Director, Hematological Malignancies/Stem Cell Transplantation Unit
David Geffen School of Medicine at UCLA, Los Angeles, California
Professor of Medicine
Division of Hematology/Oncology
Department of Medicine

Dr. Schiller is the Director of the Hematological Malignancy/ Stem Cell Transplant Program at the David Geffen School of Medicine at UCLA, supervising 200 transplants per year in 2017 and 2018. He has extensive clinical research experience, having conducted many investigator-initiated and multicenter trials, mostly in Hematologic Malignancies as well as Blood and Marrow Transplantation. He is immediate past Chair of the Faculty Executive Committee for the School of Medicine at UCLA. He is an author of over 150 publications and 270 abstracts. He has mentored residents, medical students, and fellows for more than 25 years. He has also served as a member and Chair of the Committee on Training for the American Society of Hematology and worked with its Trainee Council developing programs for the national meeting of the Society and curriculum for its Trainee Day and is presently Vice-Chair of the ASH Foundation committee. Dr. Schiller has an outstanding track record in clinical research, teaching, and mentoring and was co-investigator on Alternative Training grant for Bone Marrow Failure syndromes. He also has extensive experience outside of medicine, with nonprofit, charitable institutions. He has served on the Board of Trustees of Wilshire Boulevard Temple, and was Chairman of the Los Angeles Museum of the Holocaust. Dr. Schiller has over 25 years of experience in the diagnosis and management of adults with Hematologic Malignancies and those undergoing allogeneic stem cell transplantation for non-malignant disorders including sickle cell disease.

Akshay Sharma, MBBS
Clinical Scientist
Department of Bone Marrow Transplantation and Cellular Therapy
St. Jude Children's Hospital
Memphis, TN

Akshay Sharma is a clinician-scientist in the Department of Bone Marrow Transplantation and Cellular Therapy at St. Jude Children's Research Hospital at Memphis TN. He graduated from medical school at Kasturba Medical College in India and then pursued a postdoctoral fellowship in tumor immunology and graft engineering under the guidance of Dr. Edmund Waller at Emory University. He completed his pediatrics residency at University of Kentucky in 2015 and a pediatric hematology oncology fellowship at St. Jude in 2018. During his fellowship, he worked with Dr. Mitchell Weiss to understand the genetic regulation of fetal hemoglobin. He is currently developing novel transplant and gene therapy clinical trials for patients with sickle cell disease. The goal of his research is to advance cellular therapeutics for children with hematological disorders.

Special Guests

Mrs. Scherika Perry and Rhiannon Perry
Mother and Daughter

We are very grateful that Mrs. Perry has so willingly agreed to take part in the Webinar to share her family's experience with the Bone Marrow Transplantation that changed Rhiannon's life. We will give Rhiannon and Mrs. Perry the opportunity to share their Before and After experiences. This first hand experience will be very impactful for the patients and parents taking part in the Webinar.

Rhiannon's Story

"My name is Rhiannon Perry. I live in Manassas VA. I'm 16 and I am currently in my junior year of high school. I'm a part of the International Heritage Society, SCA, Girls Who Code, and Science Honor Society. I was diagnosed with sickle cell at birth and lupus at the age of 5. I developed a vascular necrosis in most of my joints because of the two contrasting diseases. In 2016, I had a haploidentical transplant to cure both the sickle and lupus and now at this time both diseases are cured."

Message from Putting Rare Diseases Patients First!®

We are pleased to provide this program for patients with Sickle Cell Anemia and the parents of children with Sickle Cell Anemia. We are grateful to all who support us in making this program possible. Most of all we are grateful to the patients and the groups that they have set up to provide support amongst themselves. It has been inspiring to be a part of their journeys as we have prepared for the Webinar. Do not miss hearing from the experts. Spread the word to all who are impacted by Sickle Cell Anemia, wherever they may be in the world. This will be three hours well-spent.

Link to register
https://www.puttingrarediseasespatientsfirst.org/sicklecell

The presentation will be streamed on PRDPF! FaceBook page Live

https://www.facebook.com/PuttingRareDiseasesPatientsFirst/