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Sickle Cell Disease: Advancing from past to future

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Abstract

Background and Purpose: The pathophysiology of sickle cell disease (SCD) is complex and heterogeneous. Since described by Dr. James B. Herrick in 1910, there have been improvements in the understanding of the cellular dysfunctions that occur due to hemoglobin polymerization and that lead to multi-organ damage in this disease. Novel therapies are a result of such understanding of the pathophysiology of SCD.

Methods: we conducted a PubMed search for articles published up to June 15, 2019, using the search terms "sickle cell disease," "curative therapies," "anti-oxidant," "anti-adhesive agent," "anti-inflammatory," "anticoagulant," "novel anti-sickling agents and HbF inducers," "delay production of oxyHb." Studies cited include case series, retrospective studies, prospective clinical trials, meta-analyses, online abstracts, and original reviews.

Results: The only approved long-life supportive therapy for SCD is the use of prophylactic penicillin, hydroxyurea, blood transfusion to decrease strokes and L-glutamine. However, HLA matched bone marrow transplantation is the only approved curative therapy for selected patients. The approved therapies for SCD over the last decades have improved life expectancies but have not solved many of the disease's morbidities. Thus, many clinical trials are currently being conducted for testing novel multimodality agents that target different contributing pathophysiologic processes such as: agents that target the DNA mutation defects (curative therapies), hemoglobin polymerization (anti-sickling agents) and many other targeted therapies that counter-act cellular adhesion, inflammation, oxidant injury and platelets and/or coagulation.

Conclusion: Deeper insights into the pathophysiology of SCD have led to the development of novel agents that target different contributing pathophysiologic processes.

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Speaker Biography:

Prof. Ilham Youssry, MD professor of Pediatrics, Cairo University and Head of the Pediatric Hematology and Bone Marrow Transplantation (BMT) Unit, Cairo University. Dr. Ilham received training in Pediatrics and Pediatric Hematology in New Children Hospital, Cairo University &Tokyo National Cancer Centre (NCC), Japan & Southern Illinois University, USA & The Royal London Hospital and in the BMT unit at St Mary's Hospital Paddington, UK. Professor. Ilham is an international Certified Trainer from the International Board of Certified Trainers (IBCT), Netherland. Professor Ilham received a master's degree in Health Profession Education from Maastricht University and Suez Canal University. In 2012 she completed training in a 2-year program in Foundation of Advanced International Medical Education and Research (FAIMER), Philadelphia, USA. Professor Ilham was the director of the Pediatric Hematology and Bone Marrow Transplantation (BMT) Unit, Cairo University from 2011 to 2015, during this time she initiated BMT service for a growing number of thalassemia and aplastic anemia patients. She is the supervisor of more than 50 MD & MSc theses on Thalassemia, Sickle Cell anemia, Spherocytosis, Gaucher disease, Aplastic Anemia, Thrombocytopenia and Hemophilia. She has more than 40 publications in national and international journals. Dr. Ilham was a speaker and chairperson in many national and international scientific meetings.

Abstract Citation:

Sickle cell disease is caused by a genetic abnormality in the gene for hemoglobin, which results in the production of sickle hemoglobin. When oxygen is released from sickle hemoglobin, it sticks together and forms long rods, which damage and change the shape of the red blood cell. The sickle red blood cells cause the symptoms of sickle cell disease.The sickle-shaped red blood cells break apart easily, causing anemia.

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