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THE FOUNDATION FOR

**SICKLE CELL
DISEASE
RESEARCH**

CHANGE

THE CONVERSATION.

RESHAPE

THE FUTURE.

Weekly Newsletter

February 26, 2025



Register now for the Virtual Symposium on Gene Therapy on March 13

It's an exciting time for the sickle cell disease community, thanks to groundbreaking developments in gene therapy. Join us for a one-day symposium, showcasing advancements in the fight against sickle cell disease, held Thursday, March 13, 10 a.m. to 5:30 p.m.

This virtual symposium offers a unique opportunity to gain practical insights and stay at the forefront of this transformative field. The all-day learning opportunity brings together top researchers, clinicians, and industry leaders to explore the latest innovations, challenges, and real-world applications of gene therapy. It will help medical practitioners understand how to apply existing and developing therapies to the treatment plans of current patients.

To discover how gene therapy is reshaping the future of sickle cell treatment, register today!

Explore the Topics

The Science of Gene Therapy

Mark C. Walters, M.D.

Understand the principles of gene therapy and its applications for sickle cell disease.

A Patient-Centered Approach to Accessing Curative Therapies

Julie Kanter, M.D.

Understand the two FDA-approved types of gene therapy for sickle cell disease and how healthcare providers can offer these treatments.

Gene Addition Gene Therapy and Current Limitations

Alexis Thompson, M.D., MPH

Understand the potential side effects and general concerns associated with gene addition therapy and explore fertility-related considerations in gene therapy treatments.

Gene Editing Gene Therapy and Current Limitations

Haydar Frangoul, M.D., M.S.

Learn the principles and techniques of gene editing in sickle cell and discuss the current limitations of gene editing therapies.

Access to Curative Therapies

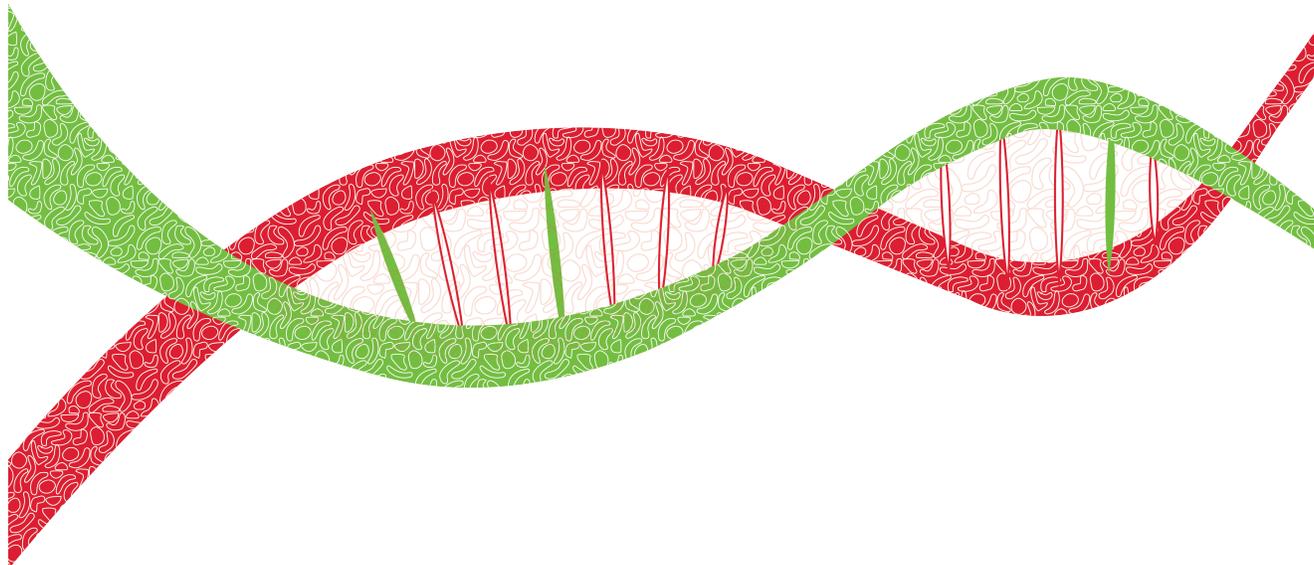
Jennifer Adair, Ph.D.

Discuss strategies for improving accessibility to treatments for underserved populations.

Future Directions of Gene Editing

Daniel E. Bauer, M.D., Ph.D.

Register for the Virtual Symposium



Dr. Walters to serve at Scientific Chair



The Foundation for Sickle Cell Disease Research is pleased to announce the new Scientific Chair for the Virtual Symposium on Gene Therapy is **Mark C. Walters, M.D.**

Dr. Walters is the Jordan Family Director of the Blood and Marrow Transplantation Program at UCSF Benioff Children's Hospital, in Oakland, California. He also acts as a professor and Chief of the Hematology Division in the Department of Pediatrics at University of California, San Francisco School of Medicine. He is Program Director of the California Institute of Regenerative Medicine Alpha Clinic at UCSF.

He has been active in cooperative clinical transplantation trials and has participated in NIH-supported investigations of hematopoietic cell transplantation for sickle cell disease and thalassemia and in industry-sponsored gene therapy clinical trials for hemoglobin disorders.

Currently, his research interests are focused on genomic editing and gene addition therapies as a strategy to extend curative therapy in all patients who inherit a clinically significant hemoglobinopathy such as sickle cell disease.

Thank you for your continued support of the FSCDR.

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