JUNE 19

SICKLE CELL DAY

Raising **awareness**
Promoting **understanding**
Inspiring **hope**

Sickle cell disease affects millions worldwide
Together, we can **support**, **advocate for** and drive **better outcomes** for every individual

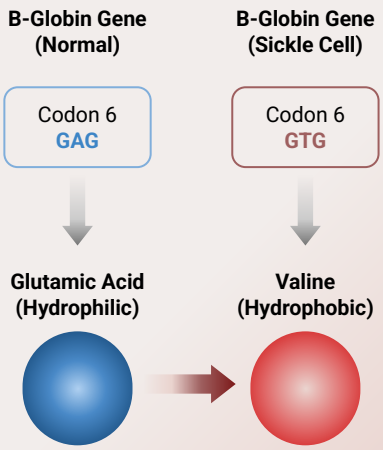
Sickle cell disease (SCD) is a group of inherited red blood cell disorders caused by a mutation in the HBB gene. It affects the shape and function of red blood cells, leading to pain, organ damage and other serious health complications

HOW IS IT CAUSED?

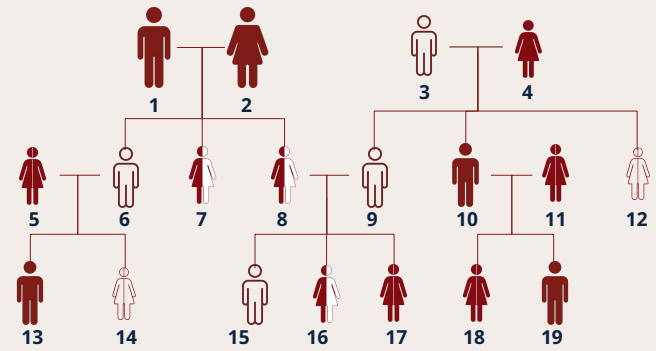
A mutation in the HBB gene causes abnormal hemoglobin (HbS). Under low-oxygen conditions, HbS causes red blood cells to become hard and sticky (sickle-shaped). These cells can block blood flow, leading to pain, anemia and organ damage

Inherited in an autosomal recessive pattern. Both parents must carry the mutated gene for a child to be affected

THE GENETIC MUTATION



PEDIGREE ANALYSIS



Key Characteristics: Skips generations, equal occurrence, carriers exist

KEY SYMPTOMS

- Recurrent pain crises (VOC)
- Chronic anemia & fatigue
- Frequent infections
- Acute chest syndrome
- Stroke & organ damage

APPROVED DRUGS FOR SICKLE CELL DISEASE

DRUG	MECHANISM OF ACTION	INDICATION	APPROVED BY FDA	COMPANY
HYDROXYUREA	Increases fetal hemoglobin (HbF), reduces sickling	Reduces pain crises and need for transfusions	1998	Various(Generic)
L-GLUTAMINE (ENDARI®)	Reduces oxidative stress in red blood cells	Reduces acute complications of SCD	2017	Emmaus
CRIZANLIZUMAB-(ADAKVEO®)	P-selectin inhibitor; reduces vaso-occlusion	Reduces frequency of pain crises	2019	NOVARTIS
CASGEVY®	CRISPR/Cas9 gene-edited autologous stem cells	For severe SCD in eligible patients (12+ years)	2023	VERTEX
LYFGENIA™	Lentiviral gene therapy to increase HbF	For severe SCD in eligible patients (12+ years)	2023	bluebirdbio

TREATMENT APPROACH

Prevent & Manage Complications
Vaccinations, infection prevention, pain management, hydration and regular check-ups

Medications
Hydroxyurea, L-glutamine, crizanlizumab, HSCT

Blood Transfusions
For severe anemia or complications like stroke

Curative Option
Hematopoietic stem cell transplant (HSCT) - the only potential cure

Note: This report is generated based on publicly available data

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Normal blood cell → **Sickle-shaped red blood cell**

Inherited in an autosomal recessive pattern. Both parents must carry the mutated gene for a child to be affected

DIAGNOSIS

Newborn Screening (NBS)
Early detection through blood test shortly after birth

Hemoglobin Electrophoresis
Detects abnormal hemoglobin (HbS) in the blood

Genetic Testing
Confirms mutation in the HBB gene

Complete Blood Count (CBC)
Helps assess anemia and other blood parameters

PREVALENCE

WORLDWIDE
~7.7M people are living with sickle cell disease worldwide
~300,000 babies are born with SCD each year

UNITED STATES
~100,000 people are living with SCD in the U.S.
SCD affects ~1 in 365 African American births

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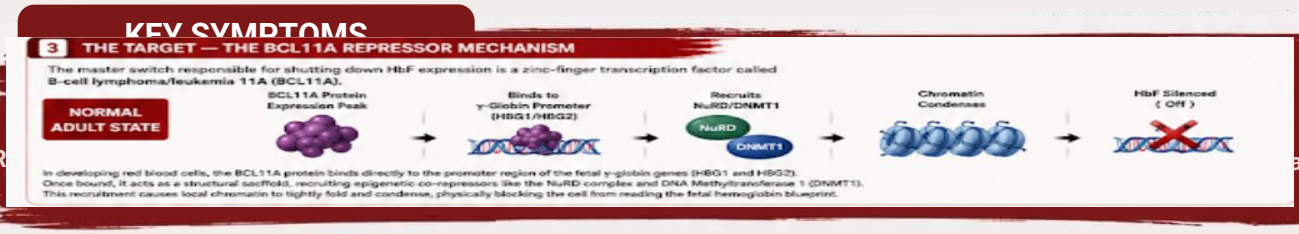

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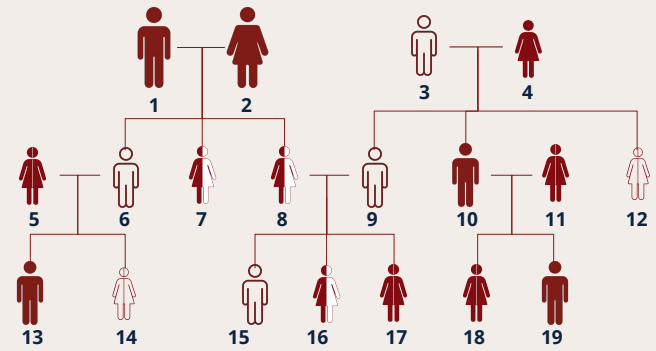
THE GENETIC MUTATION

B-Globin Gene (Normal)	B-Globin Gene (Sickle Cell)
Codon 6 GAG	Codon 6 GTG
Glutamic Acid (Hydrophilic)	Valine (Hydrophobic)

PEDIGREE ANALYSIS



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Affected: Hb^SHb^S
Carrier: Hb^AHb^S
Unaffected: Hb^AHb^A

Affected | Carrier | Unaffected

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