PRACTICE AID

Sickle Cell Disease: Resources For Your Patients[1]

Full abbreviations, accreditation, and disclosure information available at **PeerView.com/CMX40**

PeerView Hematology

Quick Facts About Sickle Cell Disease

If you or someone you know has SCD, you may have questions. This handout has answers to some common questions about SCD.

What is sickle cell disease (SCD)?
Sickle cell disease causes the body to make abnormal red blood cells. This health problem is passed down through the genes from parent to child. Normal red blood cells are soft and round. They can easily pass through tiny tubes (vessels) that carry blood around the body. Sickle cell disease causes red blood cells to be hard, pointed, and sticky—and shaped like a farm tool called a "sickle." These cells have a hard time passing through tiny blood vessels and may get stuck.

What health problems can sickle cell disease cause?
When these sickle-shaped cells block blood vessels, less blood can reach that part of the body. This can cause health problems, such as:

- Higher chance of serious infection
- Organ damage
- Attacks of severe pain
- Leg ulcers
- Stroke

Are there different types of sickle cell disease?
Sickle cell disease SS (SCD-SS) is the most common type of sickle cell disease. Some other common types are:

- SCD-SC
- SCD-S/β+ thalassemia
- SCD-S/β0 thalassemia

Sickle Cell Trait (SCT or HbAS)
People with sickle cell trait inherit one sickle cell gene ("S") from one parent and one normal gene ("A") from the other parent. People with SCT usually don't have signs of the disease and live a normal life, but they can pass the sickle cell gene on to their children. However, SCT is not a mild form of sickle cell disease.

To learn more about sickle cell disease and the sickle cell trait, contact the **Sickle Cell Disease Association of America (SCDAA)** at **1-800-421-8453** or go to **www.sicklecelldisease.org**

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Visit
https://sicklecelldisease.net/resources-educational-materials
to access the resources listed here

Sickle Cell 101

Patients Living With SCD
Health resources

Providers
MD, DO, DDS, clinical psychologist, clinical psychiatrist, nurse

Nonmedical Providers
CHWS, health navigator, social worker

Caretakers
Parent/teacher, daycare provider, etc.

Research
Data and news in the SCD community

SCD Patient Resources
Mentoring, advocacy, SCDAA initiatives

P.O.W.E.R. ECHO Project

COVID-19 Resources

Blood Drive Resources

Additional SCDAA Resources

✓ **Sickle Cell Adult Provider Network**	https://www.sicklecelldisease.org/support-and-community/providers
✓ **SCD Clinical Trial Finder**	https://sicklecelldisease.net/scdaa-trial-finder
✓ **SCDAA Patient-Powered Registry**	www.GetConnectedSCD.org
✓ **COVID-19 Information and Resources**	https://www.sicklecelldisease.org/covid-19

1. https://www.sicklecelldisease.org.

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Currently Approved and Emerging Therapies for SCD

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Therapy	Initial FDA Approval	Indications	Dosage	Most Common AEs (Incidence >10%)
Crizanlizumab[1] (Selectin blocker monoclonal antibody) *Injection*	2019	Reduction of frequency of VOCs in adults and pediatric patients with SCD aged ≥16 y	• 5 mg/kg IV infusion over 30 min on week 0, week 2, and Q4W thereafter	Nausea, arthralgia, back pain, abdominal pain, pyrexia **Warnings and Precautions** • Infusion-related reactions: Monitor for and advise patients of signs and symptoms • Interference with automated platelet counts (platelet clumping): Run test as soon as possible or use citrate tubes
Hydroxyurea[2] (Antimetabolite) *Tablets for oral use*	1967	Reduction of frequency of painful crises and need for blood transfusions in pediatric patients aged ≥2 y with SCD with recurrent moderate to severe painful crises	• Initial dose: 20 mg/kg QD; monitor blood counts Q2W • May be increased by 5 mg/kg/d Q8W, or sooner if a severe painful crisis occurs, until a maximum tolerated dose or 35 mg/kg/d is reached if blood counts are in an acceptable range	Infections, neutropenia
L-glutamine[3] (Amino acid) *Oral powder*	2017	Reduction of acute complications of SCD in adult and pediatric patients aged ≥5 y	• 5-15 g BID (based on body weight)	Constipation, nausea, headache, abdominal pain, cough, pain in extremity, back pain, chest pain
Voxelotor[4] (Hemoglobin S polymerization inhibitor) *Tablets*	2019	Treatment of SCD in adults and pediatric patients aged ≥12 y *Approved under accelerated approval based on increase in Hb; continued approval may be contingent upon verification and description of clinical benefit in confirmatory trial(s)*	• 1,500 mg PO QD • 1,000 mg PO QD in patients with severe hepatic impairment (Child Pugh C)	Headache, diarrhea, abdominal pain, nausea, fatigue, rash, pyrexia **Warnings and Precautions** • Hypersensitivity reactions: Observe for signs and symptoms and manage promptly • Laboratory test interference: Perform quantification of hemoglobin species when patient is not receiving voxelotor

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Selected Ongoing Trials in SCD[5]

Status	Study Title	Condition(s)	Intervention(s)	Phase	ClinicalTrials.gov Identifier
Recruiting	Rollover Study for Patients With Sickle Cell Disease Who Have Completed a Prior Novartis-Sponsored Crizanlizumab Study	SCD	Crizanlizumab	4	NCT04657822
Recruiting	An Indian Multi-centric Phase IV Study to Assess the Safety of Crizanlizumab in Sickle Cell Disease Patients	SCD	Crizanlizumab	4	NCT04662931
Recruiting	Study of Two Doses of Crizanlizumab Versus Placebo in Adolescent and Adult Sickle Cell Disease Patients	SCD	Crizanlizumab	3	NCT03814746
Recruiting	Study of Dose Confirmation and Safety of Crizanlizumab in Pediatric Sickle Cell Disease Patients	SCD	Crizanlizumab	2	NCT03474965
Recruiting	Study Exploring the Effect of Crizanlizumab on Kidney Function in Patients With Chronic Kidney Disease Caused by Sickle Cell Disease	SCD	Crizanlizumab Standard of care	2	NCT04053764
Active, not recruiting	Pharmacokinetics and Pharmacodynamics Study of SEG101 (Crizanlizumab) in Sickle Cell Disease (SCD) Patients With Vaso- Occlusive Crisis (VOC)	SCD	Crizanlizumab	2	NCT03264989
Recruiting	A Study to Evaluate the Safety and Efficacy of Crizanlizumab in Sickle Cell Disease Related Priapism	SCD-related priapism	Crizanlizumab	2	NCT03938454
Recruiting	A Study of FT-4202 in Adults and Adolescents With Sickle Cell Disease (HIBISCUS)	SCD	Etavopivat	2/3	NCT04624659
Active, not recruiting	A SAD/MAD to Assess the Safety, Pharmacokinetics and Pharmacodynamics of FT-4202 in Healthy Volunteers and Sickle Cell Disease Patients	SCD Healthy volunteers	Etavopivat	1	NCT03815695
Not yet recruiting	A Study of FT-4202 in Patients With Thalassemia or Sickle Cell Disease	SCD Thalassemia	Etavopivat	2	NCT04987489
Not yet recruiting	A Study Evaluating the Efficacy and Safety of Mitapivat (AG-348) in Participants With Sickle Cell Disease	SCD	Mitapivat	2/3	NCT05031780

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Selected Ongoing Trials in SCD[5] (Cont'd)

Status	Study Title	Condition(s)	Intervention(s)	Phase	ClinicalTrials.gov Identifier
Recruiting	Study to Evaluate the Effect of GBT440 on TCD in Pediatrics With Sickle Cell Disease	SCD	Voxelotor	3	NCT04218084
Recruiting	Study to Evaluate the Effect of GBT440 in Pediatrics With Sickle Cell Disease	SCD	Voxelotor	2	NCT02850406
Enrolling by invitation	Pediatric Open-Label Extension of Voxelotor	SCD	Voxelotor	3	NCT04188509
Active, not recruiting	Study to Assess the Effect of Long-term Treatment With Voxelotor in Participants Who Have Completed Treatment in Study GBT440-031	SCD	Voxelotor	3	NCT03573882
Not yet recruiting	The Effect of Voxelotor on Cerebral Hemodynamic Response in Children With Sickle Cell Anemia	SCD	Voxelotor	2	NCT05018728
Active, not recruiting	A Study Evaluating the Safety and Efficacy of bb1111 in Severe Sickle Cell Disease	SCD	bb1111	1/2	NCT02140554
Recruiting	A Study Evaluating Gene Therapy With BB305 Lentiviral Vector in Sickle Cell Disease	SCD	bb1111	3	NCT04293185
Recruiting	Study of Safety and Efficacy of Genome-edited Hematopoietic Stem and Progenitor Cells in Sickle Cell Disease (SCD)	SCD	OTQ923 HIX763 OTQ923 or HIX763	1/2	NCT04443907
Recruiting	A Study to Assess the Safety, Tolerability, Pharmacokinetics, and Pharmacodynamics of AG-946 in Healthy Volunteers and in Participants With Sickle Cell Disease	SCD Healthy volunteers	AG-946	1	NCT04536792

1. Adakveo (crizanlizumab-tmca) Prescribing Information. https://www.accessdata.fda.gov/drugsatfda_docs/label/2021/761128s001lbl.pdf. 2. Siklos (hydroxyurea) Prescribing Information. https://www.accessdata.fda.gov/drugsatfda_docs/label/2019/208843s002lbl.pdf.
3. Endari (L-glutamine) Prescribing Information. https://www.accessdata.fda.gov/drugsatfda_docs/label/2020/208587s003lbl.pdf. 4. Oxbryta (voxelotor) Prescribing Information. https://www.accessdata.fda.gov/drugsatfda_docs/label/2019/213137s000lbl.pdf. 5. https://clinicaltrials.gov.