



Frequently Asked Questions Sickle Cell Disease & Cell and Gene Therapy (CGT)

This FAQ is designed to help patients, families, and caregivers learn more about sickle cell disease, cell and gene therapy (CGT), treatment options, support services, and resources that may be available throughout the treatment journey.

Sickle Cell Disease (SCD)

Diagnosis & Basics

What is sickle cell disease?

Sickle cell disease is an inherited blood disorder that affects red blood cells. It can cause pain and other serious health complications.

What are common symptoms?

Symptoms may include:

- Pain crises (acute pain episodes)
- Chronic pain
- Fatigue
- Anemia
- Infections
- Organ complications

When is SCD diagnosed?

Most people are diagnosed at birth through newborn screening.

Treatment & Care

What treatments are available?

- Medications (such as hydroxyurea)
- Blood transfusions
- Pain management
- Preventative care

Advanced treatment options for some individuals:

- Bone marrow transplant
- Cell and gene therapy (CGT)

What is cell and gene therapy (CGT)?

CGT refers to advanced treatments that use a person's own cells or genes to treat disease. Some CGT treatments may offer the potential for long-term relief from SCD symptoms or a cure.



Cell & Gene Therapy (CGT)

Understanding CGT

Is CGT a cure for SCD?

Some CGT therapies may offer the potential for a long-term cure. Results vary from person to person, and there are benefits and risks to consider. Talk with your healthcare provider about whether CGT may be right for you.

Why do some people choose CGT?

People may consider CGT if SCD is causing frequent pain crises, hospital visits, or other serious complications. Every person's situation is different and a healthcare provider can help determine whether CGT may be an option.

Eligibility & Process

Who qualifies for CGT?

Eligibility depends on:

- Disease severity
- Overall health
- Medical history
- Provider evaluation
- Insurance criteria

What if I decide CGT is not right for me?

CGT is only one treatment option for SCD. Many people continue to manage their condition with medications, blood transfusions, preventive care, and other treatments. Talk with your healthcare team about all available options.

Where is CGT provided?

CGT is provided at specialized treatment centers, called Qualified Treatment Centers (QTCs).

What can I expect during the CGT process?

- Medical evaluation and screening
- Stem cell collection
- Laboratory modification of cells
- Chemotherapy preparation
- Infusion of modified cells
- Recovery and long-term follow-up

How long does the process take?

The entire process may take several months to a year or longer.

Risks & Recovery

What are the risks or side effects of CGT?

Possible risks and side effects may include:

- Side effects from chemotherapy
- Risk of infection
- Fatigue
- Recovery challenges

Your healthcare team can explain the potential risks and benefits based on your individual situation.

Will I need to stay in the hospital?

Yes. Most people will need a hospital stay during treatment and early recovery.

Will I still need care after CGT?

Yes. Long-term follow-up care is required after CGT.

Can CGT affect fertility?

The chemotherapy used before CGT may affect fertility. Talk with your healthcare team about fertility preservation and family planning before treatment.

How long will I be away from work, school, or daily activities?

Recovery is different for everyone. Many people need several months for treatment, recovery, and follow up care. Your healthcare team can help you understand what to expect.

Insurance, Costs & Coverage

Is CGT covered by Medicaid?

CGT may be covered by Medicaid for eligible individuals. Coverage and approval requirements can vary. Your healthcare team can help you understand your specific coverage and options.

What if my doctor is out of network?

Continuity of care protections may allow some patients to continue receiving care from their provider during treatment. Your healthcare team can help explain your options.

Do I need prior authorization?

Yes. Prior authorization is generally required before treatment can be approved.

What expenses should I plan for?

Depending on your situation, you may need help with:

- Travel and lodging
- Meals and daily living expenses
- Childcare
- Caregiver support
- Out-of-pocket medical costs

Financial assistance and support services may be available.

Healthy Living with SCD

Why is staying healthy important when considering CGT?

Good overall health can help prepare you for treatment and recovery. Your healthcare team may talk with you about nutrition, hydration, exercise, and other healthy habits as part of your care.

Is hydration important?

Yes. Staying hydrated is important for people living with SCD and may help support healthy blood flow and may reduce the risk of complications.

Support & Wraparound Services

What support services may be available?

- Transportation assistance
- Housing or lodging support
- Childcare reimbursement
- Nutrition support
- Care coordination
- Peer and patient support connections

What are wraparound services?

Wrap around services are additional supports that help patients and families successfully complete treatment and recovery.

Who helps coordinate these services?

Community-based organizations such as SCDAI, along with your healthcare team may help coordinate available services and supports.

Support System & Decision Making

What kind of support will I need?

- A caregiver or support person
- Help with transportation and appointments
- Emotional and mental health support

Why is having a support person important?

CGT often involves many appointments, hospitalization, and recovery time. Having family members, caregivers, or trusted friends available for support can help make the process easier.

Can I talk to someone who has gone through CGT?

Yes. Peer support may be available and is often encouraged for individuals and families considering treatment.

Is CGT right for me or my child?

This is a personal decision. Talk with your healthcare team, ask questions, and take time to understand the potential benefits, risks, and alternatives before making a decision.



Support is Available

Considering CGT can feel overwhelming, and it is normal to have questions. Whether you are learning about treatment options, preparing for an evaluation, or deciding if CGT is right for you or your child, support and resources are available to help you every step of the way.

Additional Questions? We're here to help.

Email: cgt@scdai.org

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