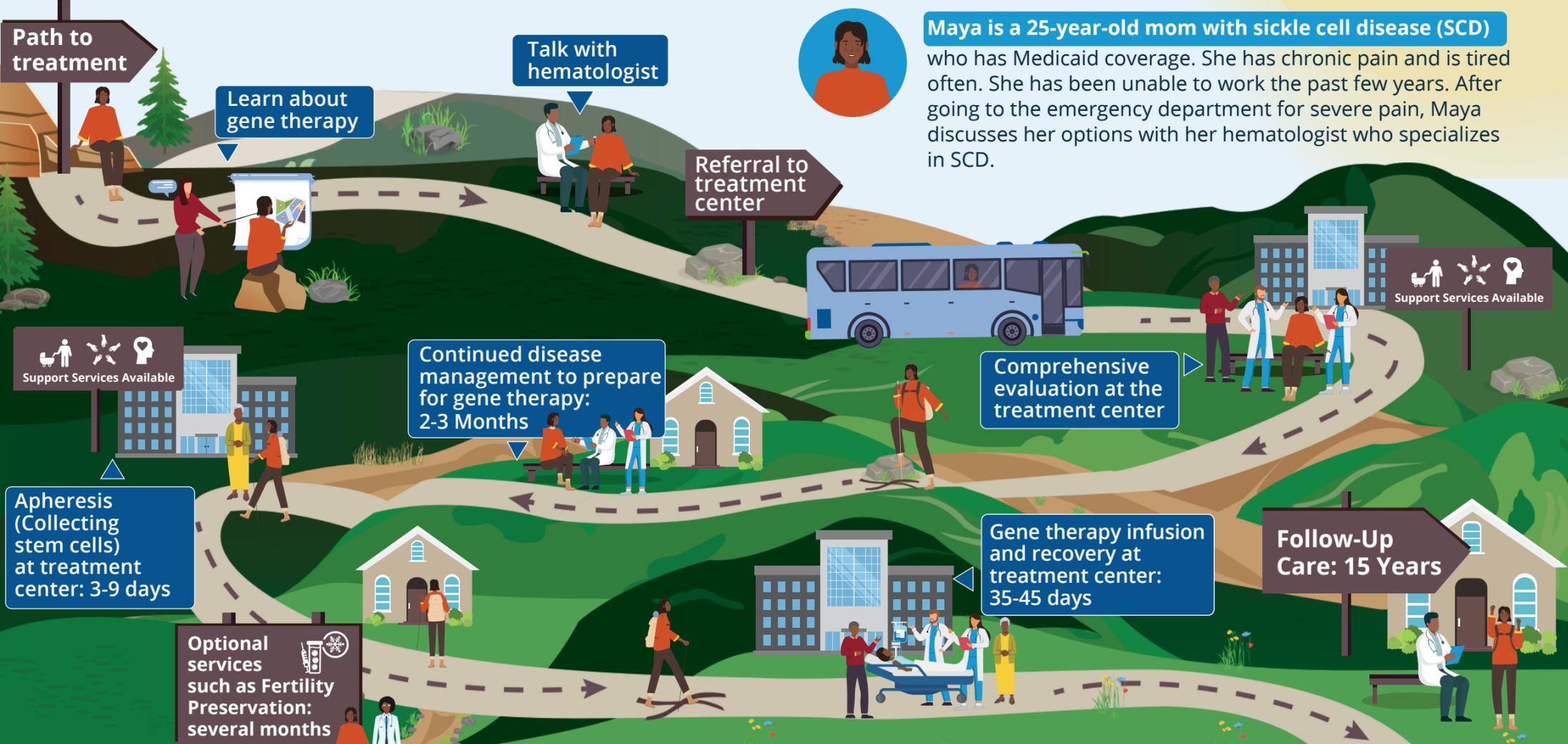


Improved Access to Gene Therapy for Sickle Cell Disease: Maya's Care Journey



Maya is a 25-year-old mom with sickle cell disease (SCD) who has Medicaid coverage. She has chronic pain and is tired often. She has been unable to work the past few years. After going to the emergency department for severe pain, Maya discusses her options with her hematologist who specializes in SCD.

Outcomes

- Access to care:** Maya receives cutting-edge, transformative treatment for SCD.
- Coordinated, whole-person care:** Maya's care team works together to support her physical and behavioral health and social needs.
- Outcomes:** Maya's quality of life vastly improves. She is able to work, enjoy her family, and engage in normal life activities.

Gene therapy is a new treatment that has great promise for people living with SCD. CMS is working together with manufacturing companies and states to make this treatment more available to people with Medicaid coverage. The graphic above shows the steps a person may go through when receiving gene therapy for SCD. Below are more details about the process and the types of medical and non-medical services that support people through this journey.

Learn about gene therapy: After a severe pain episode and conversation with her hematologist, Maya attends an informational session on gene therapy at a local community organization to learn more.

Talk with hematologist: Maya is informed that she could be a candidate for gene therapy. For further evaluation, the hematologist refers Maya to the closest treatment center, which is in a different state.

Comprehensive evaluation at the treatment center: Through Medicaid, Maya is assigned a case manager, who helps her schedule appointments. The comprehensive evaluation includes:

- Physical needs
- Behavioral health needs
- Social support needs (like transportation and travel expenses)

She meets with a SCD transplant specialist and other clinicians, including behavioral health professionals, and learns more about gene therapy for SCD, including the potential effects on her future fertility. Maya tells her care team that she may want to have more children in the future. Maya is connected to a peer support group that she continues to stay engaged with throughout her journey. After talking with her care team and family, Maya makes an informed decision to go forward with gene therapy.

Continued disease management to prepare for gene therapy: Maya is gradually taken off her disease modifying medication and begins chronic transfusion therapy for 2-3 months. Her hematologist manages her care in coordination with the team at the treatment center.

Apheresis (Collecting stem cells) at treatment center: Maya goes back to the treatment center for 3-9 days so they can harvest her stem cells. Maya's mom goes with her and mom also receives Medicaid transportation assistance and housing assistance from a community organization. Maya gets counseling and continues with her peer support group to help address her anxiety throughout the treatment process. While mom is with Maya, a community organization provides childcare for Maya's son at home.

Receive fertility preservation services: Maya returns home while her stem cells are modified, which takes several months. She gets fertility preservation services so that she has the option to have another child after her recovery from gene therapy. It consists of harvesting, freezing, and storing reproductive material.

Gene therapy infusion and recovery at treatment center: When her stem cells are ready, Maya and her mom go back to the treatment center for the longest period of treatment (35-45 days). Maya is given:

- Chemotherapy
- Gene therapy infusion
- Counseling
- Childcare and Peer support

Maya is carefully monitored by her care team throughout this period, and they develop a care plan for her return home when she is ready.

Follow-up care: Maya gets follow up care with her hematologist. She continues receiving counseling and peer support as needed. She sees her hematologist weekly for the first 6 weeks and then monthly. After the first year she has annual visits for the next 15 years. Maya's quality of life vastly improves and she is able to engage in a full range of life activities.

Contact the model team at CGTModel@cms.hhs.gov. Sign up for email updates from the CGT Model team or visit the model webpage at: cms.gov/priorities/innovation/innovation-models/cgt

