**January 2024**

# New Gene Therapies Approved for Sickle Cell Disease

## Overview

On December 8th, 2023, the FDA simultaneously approved two gene therapies — Casgevy™ and Lyfgenia™ — both indicated for the treatment of Sickle Cell Disease (SCD), a genetic blood disorder. Casgevy, priced at $2.2M, and Lyfgenia priced at $3.1M, add to the steadily expanding list of multi-million dollar gene therapies already on the market. SCD is more prevalent than the conditions targeted by other gene therapies on the market today; however, given the complex administration, cost, and availability of other treatment options, market uptake of these therapies may be initially slow. The increased potential for gene therapy claims highlights the importance of state Medicaid to develop in comprehensive strategy for managing costs while ensuring proper access to these life savings treatments.

## SCD and Market Landscape

SCD affects approximately 100,000 patients in the US. The genetic mutation present in individuals with SCD causes red blood cells, which deliver oxygen throughout the body, to become rigid and sickle shaped. This affects blood flow and may cause serious health problems, including attacks of pain (called sickle cell crisis), anemia, swelling of hands and feet, frequent infections, and damage to multiple tissues and organs. The average life expectancy of adults affected by SCD is only 52 years.

Current treatment consists of medications that act to reduce disease severity, with costs ranging from about $2,000 to $120,000 annually. Patients may also incur significant medical costs, as well as increased absenteeism. Until now, the only curative treatment has been hematopoietic stem cell transplant; however, the opportunity to receive this treatment is limited by donor availability.

Gene therapies are novel new treatments that modify or replace missing or faulty genes, potentially curing or significantly improving a chronic genetic disease. Both Casgevy and Lyfgenia are one-time infusions that require chemotherapy beforehand, and a potential lengthy hospital stay afterwards; the entire process takes about 3 to 6 months. While the therapies are similar from a clinical perspective, Casgevy's lower price point in comparison to Lyfgenia may provide a competitive market advantage. Given the complex administration, cost, and availability of other treatment options, it is possible that market uptake of these therapies may be initially slow.

## Health Equity Consideration

African Americans are disproportionately affected by SCD, with an incidence rate in the US of about 1 in 365 births. SCD patients have been a historically marginalized population, with experts indicating these patients often face systemic inequity in medical care. About 45% of SCD patients are Medicaid beneficiaries. State Medicaid programs should consider health equity when developing coverage criteria for the new gene therapies and how health disparities may impact access and pursuit of treatment in the patient population.

## Strategies for State Medicaid Programs to Consider

State Medicaid programs should evaluate how to best manage these therapies in the fee-for-service and managed care environments. In particular, states should consider the following:

- Gene therapies are administered by healthcare professionals in specially-designated treatment facilities. They require high-touch clinical support for optimal health outcomes and are typically billed through the medical benefit. In addition to the high cost of the drug itself, substantial administration and procedural fees may also come into play. States should evaluate site of service and claims payment and establish policy to ensure consistency and efficiency in payment and administration.
- Consider prior authorization and medical necessity criteria development, as appropriate. Health equity should be considered when developing coverage criteria. Additionally, states should also consider how health disparities may impact access and pursuit of treatment in the SCD patient population.
- Managed care capitation rate impact potential, in select populations.
- Risk mitigation arrangements such as risk pools, risk corridors, and/or individually carved out drugs, as appropriate.

Given that long-term data showing outcomes is still limited, it's important to recognize the potential for an incomplete clinical response or even treatment failure. State Medicaid programs should also explore the availability of outcomes-based reimbursement, while keeping in mind that these options may currently be limited and their value remains to be determined.

## For More Information

Please contact Bethany Holderread, Abby Charlier, or your Mercer pharmacy consultant to talk through the potential impact of these new therapies and updates to your specific state program. You may also email us at: mercer.government@mercer.com.

For commercial clients please contact Alina Belousova or Dulari Parikh.

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