GENE THERAPIES BRING HOPE TO PATIENTS WITH SICKLE CELL DISEASE

Sickle cell disease is caused by a mutation in the β-globin gene. This causes sickle or crescent-shaped blood cells to clog blood vessels, preventing the normal flow of nutrition and oxygen throughout the body and leading to serious complications, including pain crisis and acute chest syndrome.

50%
of patients with sickle cell disease report experiencing pain at least half the time

1 OUT OF EVERY 365
Black Americans are born with sickle cell disease

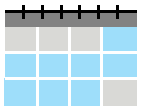
Black Americans comprise
85%
of the sickle cell disease population in the United States

Patients with sickle cell disease are hospitalized more than once a year and visit the emergency room
2 TO 3 TIMES A YEAR
most commonly due to pain crisis

The significant pain and frequent hospitalizations caused by this disease can impact employment.

Pain from sickle cell disease may force patients to:

Take unpaid time off or reduce work hours



Take a leave of absence

Stop working completely

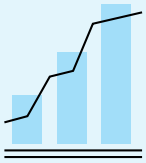
50% TO 60%
of patients with sickle cell disease report a negative impact on their employment status

Patients with sickle cell disease are estimated to earn
$750,000 LESS
over their lifetime than patients without the disease, representing a significant burden on the Black community

Potential gene therapies for sickle cell disease can reduce pain and improve a patient's quality of life.

Gene therapies in the late stages of development have demonstrated an almost complete reduction in pain crisis as well as acute chest syndrome.

Reducing these serious complications can help restore the quality of life in people with sickle cell disease and enable people to maintain more consistent and reliable employment, dramatically reducing income disparities.



$21,000
The average potential increase in income for patients in the year following a gene therapy administration for sickle cell disease

The full value of gene therapies may only be realized over a patient's lifetime. That's why our current reimbursement system needs to adapt and evolve to account for the long-term value of these therapies.

For more on the analysis, visit **PhRMA.org/Blood-Disorders**

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