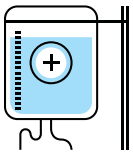
GENE THERAPIES CAN BRING HOPE TO PATIENTS

Reducing Treatment Burden and Costs

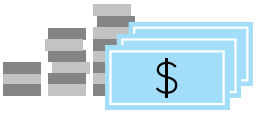


Blood disorders often require lifelong treatment involving routine drug infusions or blood transfusions. They can also be associated with frequent visits to physician offices, infusion centers or even the emergency room. Patients with these disorders often experience debilitating pain, disability, reduced life expectancy and quality of life.

Potential gene therapies may offer long-term benefits and even cures for patients with blood disorders with a single administration. Since they may drastically reduce the burden of the current standard of care, they may also:

Reduce the need for patients to miss work for treatment and/or symptoms, leading to economic benefits for patients

Dramatically reduce existing treatments for patients



Result in savings for the health care system

	HEMOPHILIA A	HEMOPHILIA B	BETA THALASSEMIA
Current Standard of Care for Patients with Severe Disease	Factor replacement therapy, generally infused 2 to 3 times a week	Factor replacement therapy, generally infused 2 to 3 times a week	17 blood transfusions a year, on average
Current Annual Health Care Costs Per Patient	As much as $760,000	$615,000	$125,000
▼ **INTRODUCTION OF A GENE THERAPY** ▼			
Benefits of Gene Therapy	Almost complete reduction in factor replacement therapy	Almost complete reduction in factor replacement therapy	Elimination of dependence on blood transfusions
Potential Savings to the Health Care System the Year Following Gene Therapy Per Patient	As much as $730,000	As much as $600,000	As much as $125,000
Potential Annual Increase in Patient Income From Greater Workforce Participation	As much as $9,500	As much as $7,000	As much as $4,000

**Figures reflect average costs, rounded up.*

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

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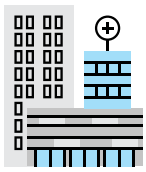
HEALTH EQUITY CASE STUDY: SICKLE CELL DISEASE

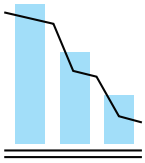
The Disease

Sickle cell disease is a rare blood disorder affecting 1 out of every 365 Black or African Americans. The disease causes crescent-shaped blood cells to clog blood vessels, which prevents the normal flow of nutrition and oxygen throughout the body and can lead to serious complications, including pain crisis and acute chest syndrome.



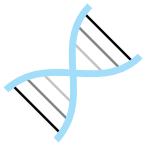
The Health Impact on Patients

Patients are hospitalized more than once a year, on average, and visit the emergency room 2 to 3 times a year, most commonly due to pain crisis. For people living with sickle cell disease, 50% report pain on half of their days and 30% report pain most of the time.



The Economic Impact on Patients

50% to 60% of patients reported the disease negatively impacted their employment status, forced them to stop working completely or take a leave of absence, or led to unpaid time off or reduced work hours. As a result, patients are estimated to **earn $750,000 less over a lifetime**, representing a significant burden on the Black and African American community disproportionately impacted by this disease.



The Treatments in the Pipeline

Gene therapies in the late stages of development have demonstrated an almost complete reduction in pain crisis.

The Benefit to Patients

By virtually eliminating the impact of this painful disease, gene therapy has the potential to dramatically reduce income disparities, leading to as much as **$21,000 more in average income** in the year following gene therapy administration alone.

While many gene therapies are administered just one time with long-lasting or even potentially curative effects, the full value they provide to patients, caregivers and the entire health care system may only be realized over the lifetime of the patient. Addressing the unique challenges presented by these groundbreaking therapies that offer the potential to transform health care requires a rethinking of the current reimbursement system to adapt and evolve to account for the long-term value these therapies provide.

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