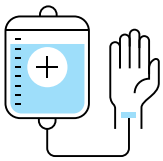
GENE THERAPIES COULD IMPROVE TREATMENT AND LOWER COSTS FOR PATIENTS WITH BETA THALASSEMIA

Beta thalassemia is a rare and inherited blood disorder that impacts red blood cells by reducing the amount of oxygen carried throughout the body.

2,000 AMERICANS
are affected with the disease

50%
of patients have severe disease



The current standard of treatment for severe beta thalassemia requires an average of

17 BLOOD TRANSFUSIONS A YEAR

which lead to various side effects, like iron overload.

Patients with severe beta thalassemia face significant costs and complications from blood transfusions, the current standard of treatment.

The average annual health care costs for patients with severe beta thalassemia are estimated at **$125,000** primarily due to regular blood transfusions and therapy to manage the iron overload side effects.

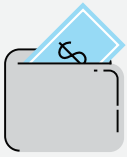
Patients with severe beta thalassemia often die by **30 years old** due to cardiac complications of iron overload from the current standard of treatment.

Gene therapies have the potential to eliminate these costs in the healthcare system and improve the quality of life for beta thalassemia patients.

GENE THERAPIES
in the late stages of development could free patients from regular blood transfusions and medicines to manage side effects of treatment in the years following a single gene therapy administration.

Therefore, gene therapies could:

Result in as much as
$125,000 IN SAVINGS
the year following administration.



Increase patient and caregiver income by as much as
$4,000 ANNUALLY
by allowing patients to avoid missed workdays due to eliminated transfusions for patients.

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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