

GENE THERAPIES COULD REDUCE TREATMENT COSTS FOR PATIENTS WITH HEMOPHILIA B



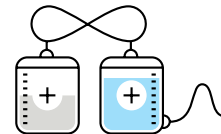
Hemophilia B is a rare genetic bleeding disorder characterized by insufficient levels of a blood protein called factor IX, which the body needs for blood clotting. Patients experience spontaneous bleeding and progressive joint damage which can lead to functional impairment, disability, poor quality of life and loss of productivity and employment.



Each year nearly
100 BABIES
are born with the disease



50%
of patients have
severe disease



Severe hemophilia B patients often require life-long prophylactic infusions of factor replacement therapy

2 TO 3 TIMES A WEEK

This current standard of treatment is burdensome and associated with a tremendous cost of care.



Average annual health care costs for patients with severe hemophilia B are
\$615,000 with more than 99% of such costs attributed to factor replacement therapy.

Gene therapies have the potential to dramatically reduce or eliminate these costs in the healthcare system and improve the quality of life for patients with hemophilia B.



GENE THERAPIES

in the late stages of development have significantly reduced bleeding rates and almost completely eliminated the need for factor replacement therapy in the years following a one-time administration.

Therefore, gene therapies could:



Result in as much as
\$600,000 IN SAVINGS
the year following a single administration.



Increase patient and caregiver income by as much as
\$7,000 ANNUALLY
by allowing patients to avoid hemophilia-related unemployment and early retirement.

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.