GENE THERAPIES COULD REDUCE TREATMENT COSTS FOR PATIENTS WITH HEMOPHILIA A

Hemophilia A is a rare genetic bleeding disorder characterized by insufficient levels of a blood protein called factor VIII, 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.

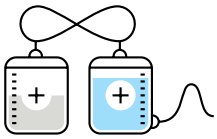
Each year nearly

400 BABIES

are born with the disease

50%

of patients have severe disease



Severe hemophilia A 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 hemophilia A treated prophylactically range as high as **$760,000** with 96% 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 A.

GENE THERAPIES

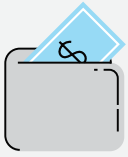
in the late stages of development have significantly reduced bleeding rates and almost completely eliminated the need for factor replacement therapy in the years following a one-time administration.

Therefore, gene therapies could:

Result in as much as

$730,000 IN SAVINGS

the year following a single administration.



Increase patient and caregiver income by as much as

$9,500 ANNUALLY

by allowing patients to avoid hemophilia-related under-employment 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.

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

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