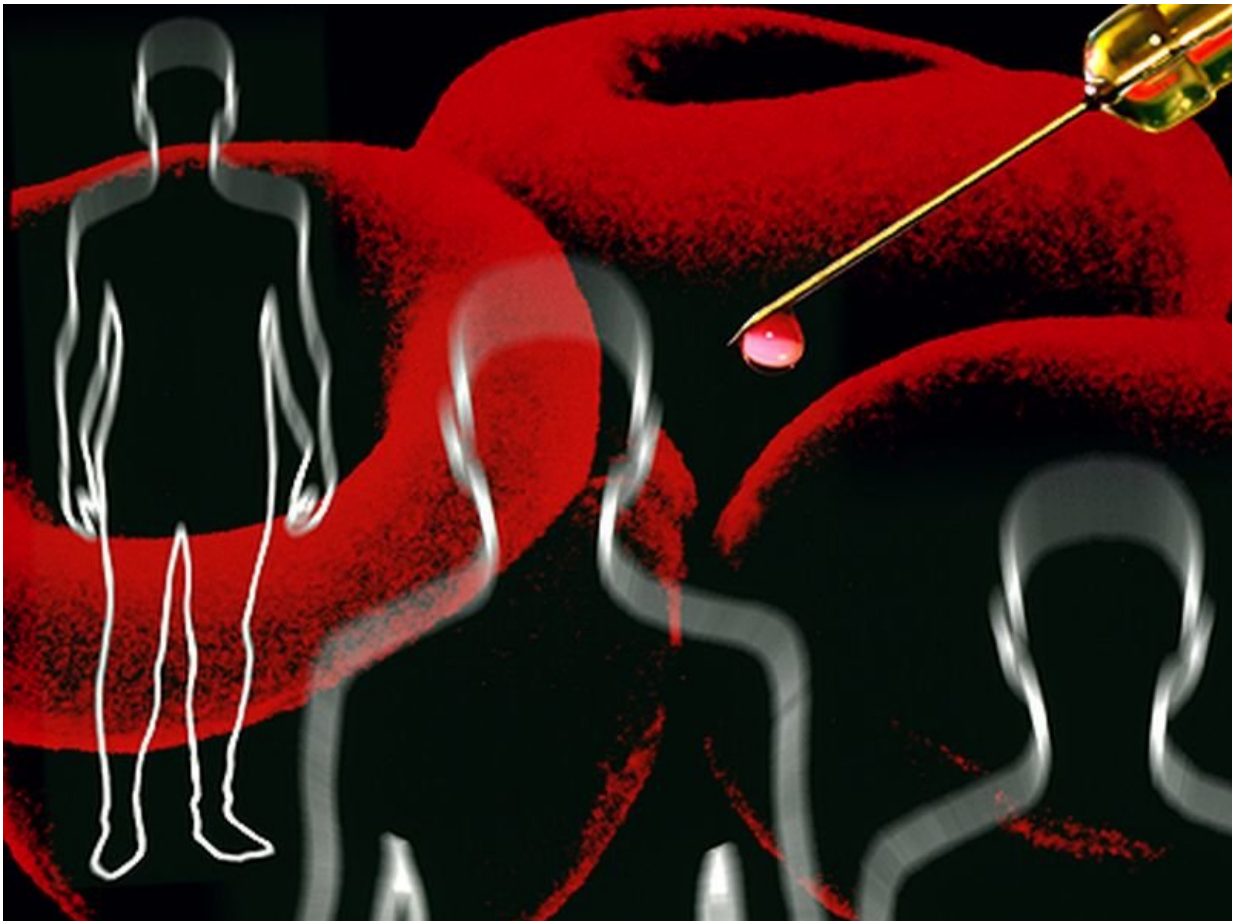


ASH: High-dose gene transfer beneficial in severe hemophilia A

December 11 2017



(HealthDay)—For men with severe hemophilia A, high-dose factor VIII

gene transfer is associated with sustained normalization of factor VIII activity levels, according to a study published online Dec. 9 in the *New England Journal of Medicine* to coincide with the annual meeting of the American Society of Hematology, held from Dec. 9 to 12 in Atlanta.

Savita Rangarajan, M.B., B.S., from Hampshire Hospitals NHS Foundation Trust in Basingstoke, U.K., and colleagues infused a single intravenous dose of a codon-optimized adeno-associated virus serotype 5 (AAV5) vector encoding a B-domain-deleted human factor VIII (AAV5-hFVIII-SQ) in nine men with severe hemophilia A. Participants were enrolled into one of three dose cohorts (low dose [one participant], intermediate dose [one participant], and high dose [seven participants]).

The researchers found that recipients of the low or intermediate dose had factor VIII [activity levels](#) that remained at 3 IU or less/dL. In all seven participants in the high-dose cohort, the factor VIII activity level was more than 5 IU/dL between weeks two and nine after [gene transfer](#); in six participants the level increased to a normal value (>50 IU/dL), which was maintained at one year after receipt.

"The infusion of AAV5-hFVIII-SQ was associated with the sustained normalization of factor VIII activity level over a period of one year in six of seven participants who received a [high dose](#)," the authors write.

The study was funded by BioMarin Pharmaceutical.

More information: [Abstract](#)

[Full Text](#)

[Editorial](#)

[More Information](#)

Copyright © 2017 [HealthDay](#). All rights reserved.

Citation: ASH: High-dose gene transfer beneficial in severe hemophilia A (2017, December 11)
retrieved 27 April 2024 from

<https://medicalxpress.com/news/2017-12-ash-high-dose-gene-beneficial-severe.html>

This document is subject to copyright. Apart from any fair dealing for the purpose of private study or research, no part may be reproduced without the written permission. The content is provided for information purposes only.