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

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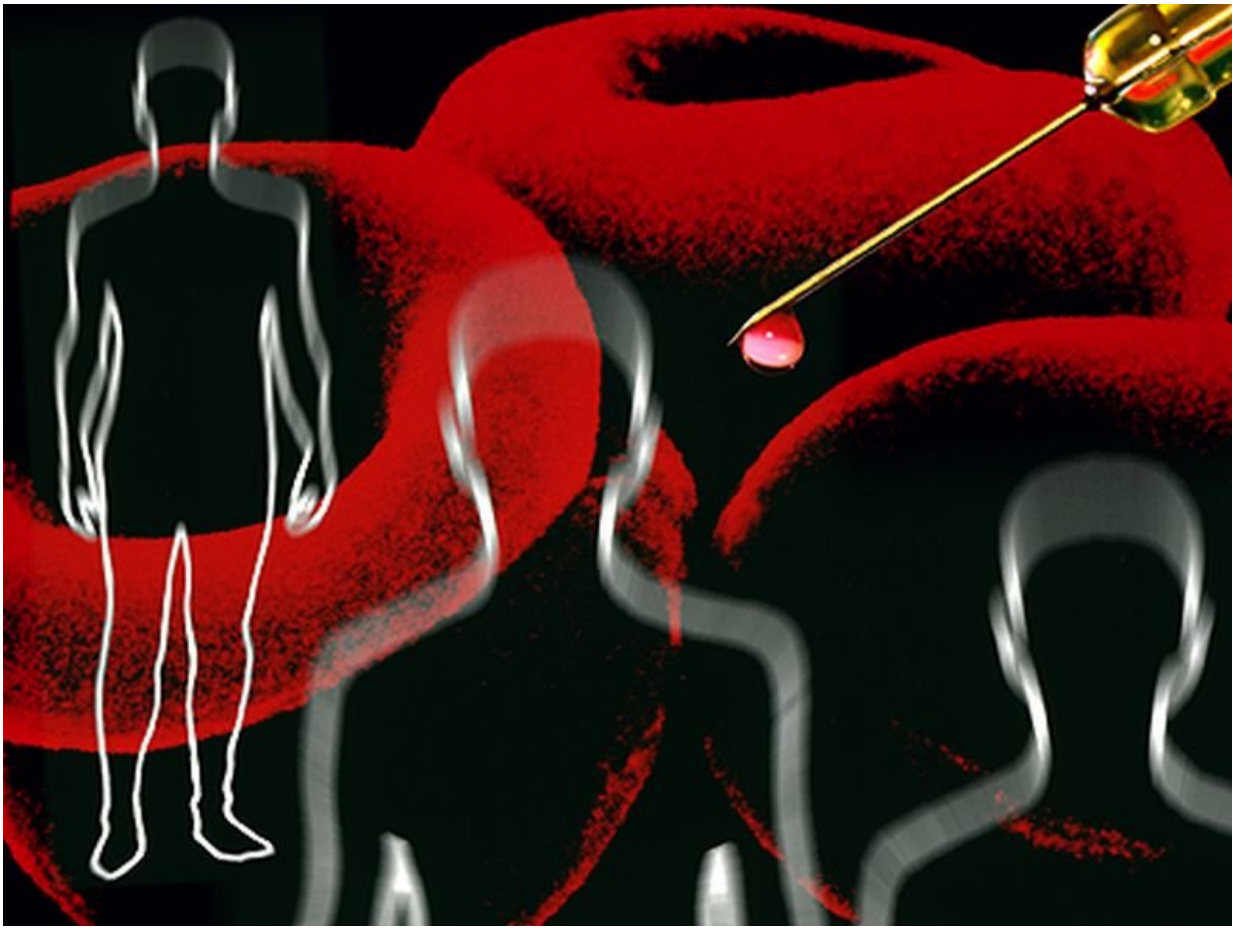

(HealthDay)—For men with severe hemophilia A, high-dose factor VIII

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.

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