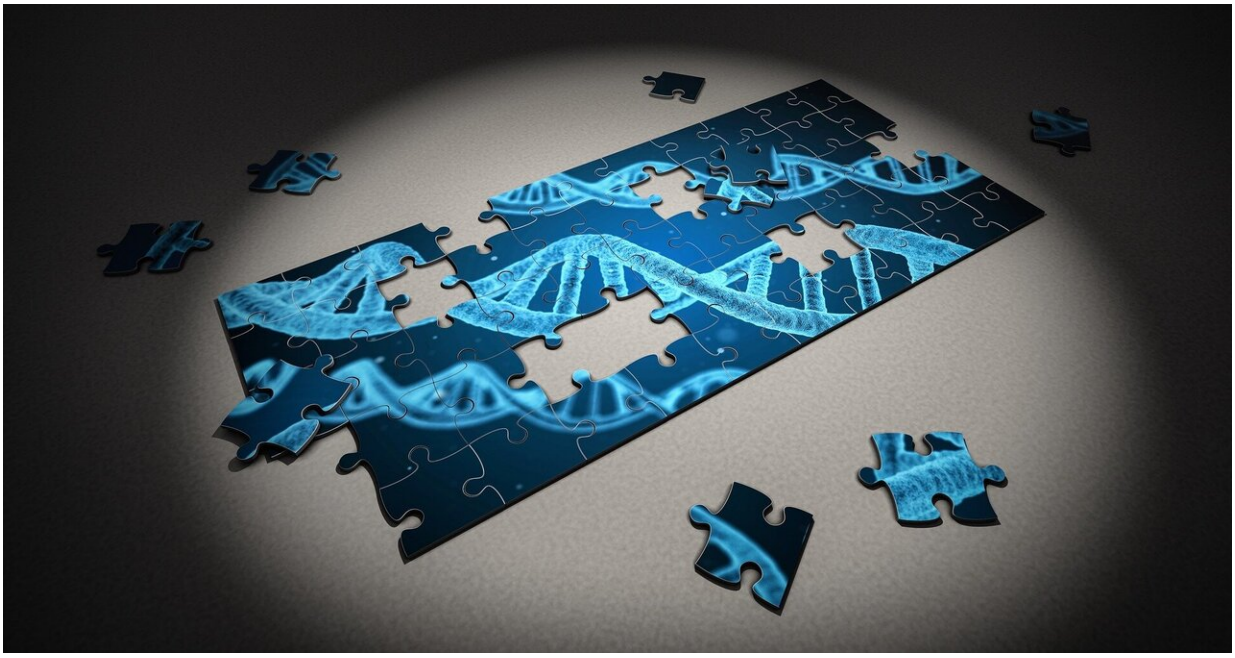
MedicalXpress

Gene therapy shown to offer long-term benefits for people with Haemophilia A

January 6 2020



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A breakthrough gene therapy treatment for Haemophilia A has been shown to offer long-term benefits that have already transformed the lives of 13 men in the UK.

Clinical researchers led by Professor John Pasi from Queen Mary University of London first announced in 2017 that 85% of men treated

with a single infusion of a missing gene were showing normal or near-normal levels of the blood-clotting protein, factor VIII, one year on.

In a new paper published in the *New England Journal of Medicine*, Professor Pasi, who is also Director of the Haemophilia Centre at Barts Health NHS Trust, confirmed that all the patients involved in the trial were still benefiting from a substantial fall in the rates of bleeding three years after receiving the treatment. None of the 13 patients had required regular factor VIII to prevent bleeding during that period.

A lack of factor VIII causes Haemophilia A, which accounts for around 80 per cent of all Haemophilia cases. With the blood unable to clot, patients are at risk of excessive bleeding from even the slightest injury—as well as potentially life-threatening spontaneous internal bleeding. Patients have to undergo three or more intravenous injections each week to control and prevent bleeding. Eliminating these regular injections greatly reduces the burden of treatment needed to keep bleeding at bay.

Professor Pasi said: "Our 2017 paper showed that gene therapy could significantly boost factor VIII levels in men with Haemophilia A. Our new data are critical in helping the scientific and medical communities understand this pioneering technology. This latest study confirms both safety and the long-term beneficial impact of the treatment. A long-term treatment that effectively ends the life-long regime of regular injections can transform care and massively improve the quality of life of hundreds of thousands of people born with this challenging genetic condition."

The treatment could be particularly important in the developing world where access to clotting products is difficult.

More information: K. John Pasi et al, Multiyear Follow-up of AAV5-hFVIII-SQ Gene Therapy for Hemophilia A, *New England*

Journal of Medicine (2020). DOI: 10.1056/NEJMoa1908490