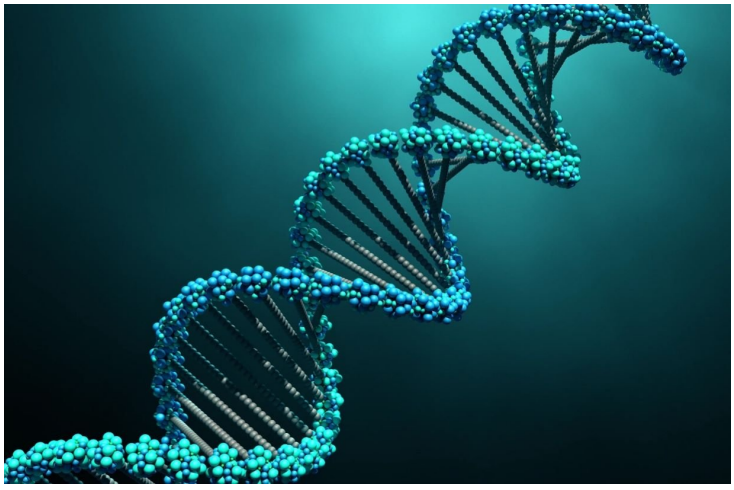
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DBT supports first-in-human gene therapy using lentiviral vectors for severe hemophilia A

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Marks a transformative leap in accessible and effective treatments for resource-limited settings



In a note-worthy scientific achievement for medical science in India, the first-in-human gene therapy using lentiviral vectors for severe hemophilia A has demonstrated transformational results.

Supported by the Department of Biotechnology, this innovative therapy was developed by the Centre for Stem Cell Research (CSCR) at Christian Medical College (CMC), Vellore, a translational unit of BRIC-inStem.

The single-centre study has successfully produced zero annualised bleeding rates in all five enrolled participants while enabling prolonged production of Factor VIII, eliminating the need for repeated infusions.

Hemophilia A, a severe bleeding disorder caused by the deficiency of clotting Factor VIII, significantly affects patients' quality of life, leading to spontaneous bleeding episodes. Although rare, India bears the world’s second-largest burden of hemophilia, with approximately 136,000 cases. Current treatments require frequent Factor VIII replacement therapy, which faces challenges such as high costs, venous access in children, and low patient acceptance.

The gene therapy approach developed by CSCR involves the use of a lentiviral vector to introduce a normal copy of the Factor VIII gene into autologous hematopoietic stem cells (HSCs). These modified HSCs generate blood cells capable of producing functional Factor VIII over extended periods. The trial, involving five participants, observed zero annualized bleeding rates over a cumulative follow-up of 81 months, correlating Factor VIII activity with vector copy numbers in the peripheral blood.

Participants were monitored for six months following the gene therapy. Results showed a strong correlation between Factor VIII activity levels and the vector copy number in peripheral blood. Remarkably, all five participants achieved a zero annualized bleeding rate, sustained over a cumulative follow-up period of 81 months. This achievement underscores the long-term efficacy and safety of the therapy, offering renewed hope for patients with severe Hemophilia A.