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India conducts first human clinical trial of gene therapy for haemophilia A at CMC Vellore

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Trials involved deploying a novel technology of using a lentiviral vector



India has conducted the first human clinical trial of gene therapy for haemophilia A (FVIII deficiency) at Christian Medical College (CMC) Vellore. This was disclosed by Union Minister of Science & Technology Dr Jitendra Singh while addressing the "National Science Day 2024" programme in Vigyan Bhavan in New Delhi, on 28 February 2024.

Dr Jitendra Singh further informed that the programme is supported by the Department of Biotechnology, the Centre for Stem Cell Research - a unit of InStem Bengaluru, in collaboration with Emory University, USA at Christian Medical College, Vellore.

The trials involved deploying a novel technology of using a lentiviral vector to express a FVIII transgene in the patient’s own haematopoietic stem cell which will then express FVIII from specific differentiated blood cells.

The Minister expressed the hope that manufacturing of this vector will commence soon in India and proceed with further clinical trials.