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European Union approves first gene therapy for hemophilia B

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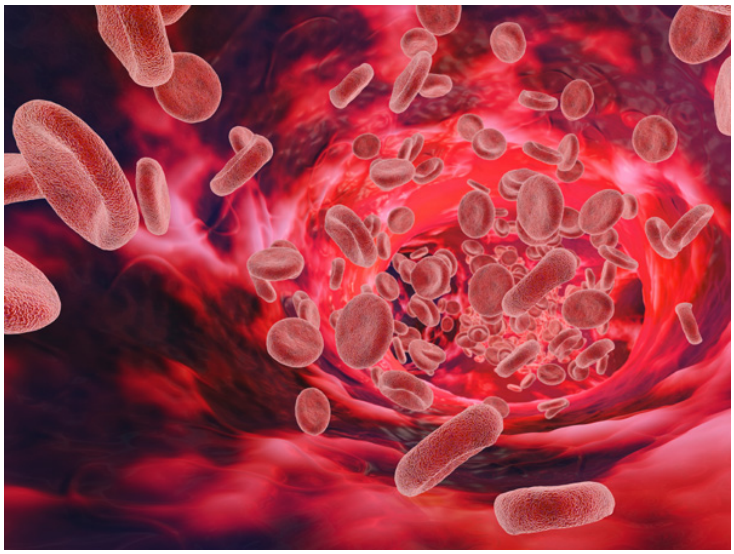
European Commission approves Autralian firm CSL's gene therapy for Hemophilia B



Australian biotechnology leader CSL has announced that the European Commission has granted conditional marketing authorisation (CMA) for HEMGENIX (etranacogene dezaparvovec), the first and only one-time gene therapy for the treatment of severe and moderately severe hemophilia B (congenital Factor IX deficiency) in adults without a history of Factor IX inhibitors. It is the first approved gene therapy for hemophilia B in the European Union (EU) and European Economic Area (EEA).

The European Commission's decision follows the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP)'s positive opinion in December 2022, based on findings from the pivotal HOPE-B trial, the largest gene therapy trial in hemophilia B to date.

HEMGENIX is an adeno-associated virus five (AAV5)-based gene therapy given as a one-time treatment for moderately severe to severe hemophilia B patients.

This approval marks an important step forward in the treatment of hemophilia B, which changes the lives of people weakened by bleeding in the muscles, joints and internal organs, alleviating the burden of lifelong intravenous infusions of Factor IX products. Data from the HOPE-B study demonstrated the potential of HEMGENIX to remove the need for routine prophylaxis, by providing durable Factor IX activity, as well as improved bleeding outcomes and quality of life for people with hemophilia B.