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CSL hemophilia therapy gets orphan drug status

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CSL Hemophilia therapy gets FDA orphan drug designation

Singapore: CSL Behring's novel recombinant fusion protein linking coagulation factor IX with recombinant albumin (rIX-FP), which can be used for the treatment of Hemophilia B, has been granted orphan drug designation by the US FDA.

The recombinant protein can be used for the treatment and prophylaxis of bleeding episodes in patients with congenital factor IX deficiency. The designation includes routine prophylaxis treatment, control and prevention of bleeding episodes, and prevention and control of bleeding in perioperative settings. The fusion protein has been engineered to extend the half-life of factor IX through genetic fusion with recombinant albumin.

CSL Behring, which is a subsidiary of Melbourne-based CSL Limited and a leader in plasma protein therapeutics, is developing the therapy in collaboration with its parent company.

Dr Val Romberg, senior VP, R&D, CSL Behring, said that, "CSL Behring is pleased to have achieved this important regulatory milestone for our recombinant factor IX. It represents yet another advance that our company is making in the area of recombinant factor development and is extremely encouraging."

The FDA's orphan drug designation program provides orphan status to unique drugs and biologics, defined as those intended for the safe and effective treatment or prevention of rare diseases that affect fewer than 200,000 people in the US.