FDA approves treatment for rare blood clotting disorder

January 2024—The Food and Drug Administration approved Adzynma (Takeda Pharmaceuticals), the first recombinant protein product indicated for prophylactic or on-demand enzyme replacement therapy in adult and pediatric patients with congenital thrombotic thrombocytopenic purpura (cTTP), a rare and life-threatening blood clotting disorder. Treatment for cTTP typically involves prophylactic plasma-based therapy for people with chronic disease to reduce the risk of clotting and bleeding by replenishing the absent or low ADAMTS13 enzyme. Adzynma is a purified recombinant form of the ADAMTS13 enzyme that works by providing a replacement for the low levels of the deficient enzyme in patients with cTTP. For prophylactic ERT, Adzynma is administered to help reduce the risk of disease symptoms. The product may also be administered as an on-demand ERT for treatment when the patient is experiencing an acute event.

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