FDA approves first treatment for congenital thrombotic thrombocytopenic purpura

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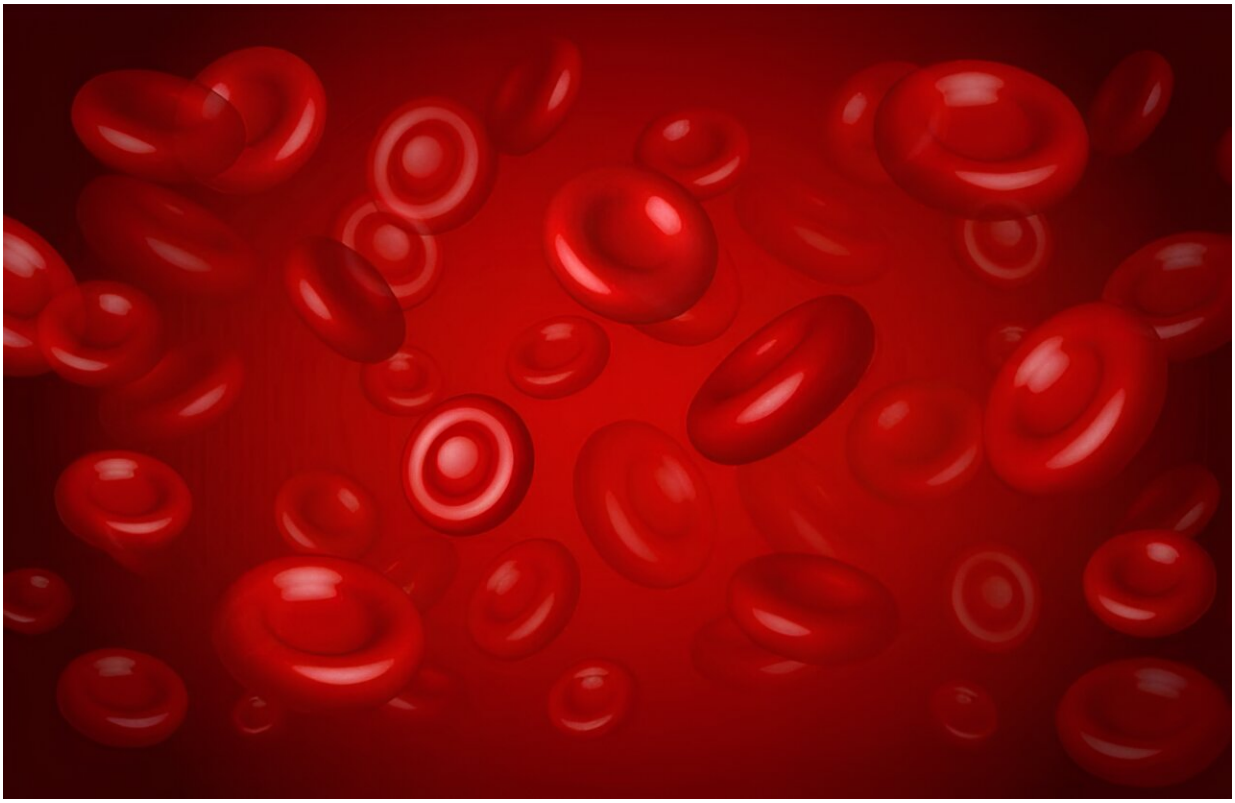

The U.S. Food and Drug Administration has approved Takeda Pharmaceuticals Adzynma, the first recombinant protein product indicated for prophylactic or on-demand enzyme replacement therapy (ERT) in adult and pediatric patients with congenital thrombotic

thrombocytopenic purpura (cTTP).

Adzynma is a purified recombinant form of the ADAMTS13 enzyme that enhances the low levels of the deficient enzyme in patients with cTTP. For prophylactic ERT, Adzynma is administered intravenously once every other week; for on-demand ERT, it is administered once daily. The most common side effects included headache, diarrhea, migraine, abdominal pain, nausea, upper respiratory tract infection, dizziness, and vomiting.

The priority review, fast track, and orphan-designated approval was based on results from a trial in which 46 patients were randomly assigned to receive six months of treatment with either Adzynma or plasma-based therapies (period 1), then crossed over to the other treatment for six months (period 2). Effectiveness of Adzynma was determined based on the incidence of TTP events and TTP manifestations, as well as the incidence of the need for supplemental doses.

"Today's approval reflects important progress in the development of much-needed treatment options for patients affected by this life-threatening disorder," Peter Marks, M.D., Ph.D., director of the FDA Center for Biologics Evaluation and Research, said in a statement. "The FDA remains deeply committed in our efforts to help facilitate the development and approval of safe and effective therapies for patients with rare diseases."

More information: More Information

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