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ASIA EDITION

FDA approves Sanofi's blood disorder drug

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First medicine approved in the U.S. specifically for the treatment of aTTP, a rare blood-clotting disorder

The U.S. Food and Drug Administration (FDA) has approved Cablivi (caplacizumab-yhdp) in combination with plasma exchange and immunosuppression for the treatment of acquired thrombotic thrombocytopenic purpura (aTTP) in adults. Cablivi is the first FDA approved therapy specifically indicated for the treatment of aTTP.

"The U.S. approval of Cablivi provides a much-needed treatment option for people facing this challenging disease. There have been limited medicines available to treat aTTP until now," says Olivier Brandicourt, M.D., Chief Executive Officer, Sanofi. "Cablivi marks the first U.S. approval in our newly formed rare blood disorders franchise, and we look forward to continuing to provide important medicines for people living with these very serious diseases."

Cablivi targets von Willebrand factor (vWF), a protein in the blood involved in hemostasis. It is designed to inhibit the interaction between vWF and platelets. Cablivi is an anti-vWF Nanobody and Sanofi's first Nanobody®-based medicine to receive approval in the U.S. Nanobodies are a novel class of proprietary therapeutic proteins based on single-domain antibody fragments that contain the unique structural and functional properties of naturally-occurring heavy chain only antibodies.

Cablivi received FDA Fast Track designation and was evaluated under Priority Review, which is reserved for medicines that represent significant improvements in safety or efficacy in treating serious conditions.

An Unmet Need in a Rare Blood Disorder

aTTP is a rare, life-threatening, autoimmune blood disorder. aTTP is considered an urgent, medical emergency. For some patients, resuscitative measures might be required and the immediate outcome might not be predictable. In most cases, patients are routinely treated in intensive care units during the first few days following their aTTP diagnosis. It is estimated that up to 20% of patients die from TTP episodes, despite currently available treatments (plasma exchange and immunosuppression), with most deaths occurring within 30 days of diagnosis. In the U.S., aTTP affects fewer than 2,000 adults each year.

"aTTP is a very severe, life-threatening disease. For those faced with this rare diagnosis, the treatment and care can be difficult and the threat of recurrence is ever-present," said Spero R. Cataland, M.D., Professor of Internal Medicine, Division of Hematology, Wexner Medical Center at the Ohio State University. "Cablivi provides new hope for adults in the U.S. suffering with aTTP and provides a much needed treatment option to help effectively manage aTTP episodes."

In aTTP, accumulation of ultra-large vWF causes extensive clot formation in small blood vessels throughout the body, leading to severe thrombocytopenia (very low platelet count), microangiopathic hemolytic anemia (loss of red blood cells through destruction), and ischemia (restricted blood supply to parts of the body).