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Cablivi gets FDA approval to treat rare blood clotting disorder

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The U.S. Food and Drug Administration has approved Cablivi (caplacizumab-yhdp) injection, the first therapy specifically indicated, in combination with plasma exchange and immunosuppressive therapy, for the treatment of adult patients with acquired thrombotic thrombocytopenic purpura (aTTP), a rare and life-threatening disorder that causes blood clotting.

Patients with aTTP develop extensive blood clots in the small blood vessels throughout the body. These clots can cut off oxygen and blood supply to the major organs and cause strokes and heart attacks that may lead to brain damage or death. Patients can develop aTTP because of conditions such as cancer, HIV, pregnancy, lupus or infections, or after having surgery, bone marrow transplantation or chemotherapy.

The efficacy of Cablivi was studied in a clinical trial of 145 patients who were randomized to receive either Cablivi or a placebo. Patients in both groups received the current standard of care of plasma exchange and immunosuppressive therapy. The results of the trial demonstrated that platelet counts improved faster among patients treated with Cablivi, compared to placebo.

Treatment with Cablivi also resulted in a lower total number of patients with either aTTP-related death and recurrence of aTTP during the treatment period, or at least one treatment-emergent major thrombotic event (where blood clots form inside a blood vessel and may then break free to travel throughout the body).

The proportion of patients with a recurrence of aTTP in the overall study period (the drug treatment period plus a 28-day follow-up period after discontinuation of drug treatment) was lower in the Cablivi group (13 percent) compared to the placebo group (38 percent), a finding that was statistically significant.

The FDA granted this application Priority Review designation. Cablivi also received Orphan Drug designation, which provides incentives to assist and encourage the development of drugs for rare diseases.