Caplacizumab

National Cancer Institute

Source

National Cancer Institute. Caplacizumab. NCI Thesaurus. Code C128625.

A humanized, bivalent anti-von Willebrand factor (VWF) nanobody, with potential anti-platelet and anti-thrombotic activities. Upon administration, caplacizumab specifically binds, with its two identical monovalent moieties, to the A1 domain of the adhesive glycoprotein VWF, thereby inhibiting and neutralizing VWF activity. This prevents the interaction of ultra-large VWF (ULVWF) with the platelet glycoprotein (GP)Ib-IX-V receptor complex, and prevents ULVWF-mediated platelet adhesion, and aggregation, which reduces thrombus formation. VWF is a glycoprotein and plays a key role in blood coagulation. Increased VWF, which is seen in a number of diseases, is associated with an increased risk in thrombosis; in thrombotic thrombocytopenic purpura (TTP), increased levels of ULVWF and thus increased and abnormal platelet aggregation are seen due to impaired breakdown of ULVWF. The nanobody formulation allows for rapid distribution, onset of action and clearance. The nanobody is based on the smallest functional fragments of the immunoglobulin heavy-chain variable domains that occur naturally in the Camelidae family.