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Complement Factor D Inhibitor ACH-4471

National Cancer Institute

Source

National Cancer Institute. <u>Complement Factor D Inhibitor ACH-4471</u>. NCI Thesaurus. Code C148181.

An orally bioavailable inhibitor of complement factor D (FD; CFD), a serine protease that cleaves complement factor B, with potential complement system inhibiting activity. Upon administration, complement FD inhibitor ACH-4471 targets, binds to and blocks the activity of FD, and thereby inhibits cleavage of complement factor B into Ba and Bb in the alternative pathway of the complement cascade. T his inhibits FD-mediated signaling and activation of the alternative complement pathway (ACP), blocks complement-mediated hemolysis in paroxysmal nocturnal hemoglobinuria (PNH) and prevents ACP-induced tissue damage. FD plays a key role in the activation of the ACP.