

Data Sheet

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Global Supplier of Chemical Probes, Inhibitors & Agonists

Product Name :Danicopan (Synonyms: ACH-4471)

Molecular Weight :580.41

 $\begin{tabular}{ll} \textbf{Molecular Formula} & : C_{26}H_{23}BrFN_7O_3 \\ \end{tabular}$

Target : Solubility :

O HN O F

Biological Activity

Danicopan(ACH-4471) is an effective and selective inhibitor of complement factor D, with an IC50 value of 0.6 nM. Complement factor D is a serine protease that cleaves factor B, which activates the alternative complement pathway. Danicopan(ACH-4471) has been demonstrated to inhibit the alternative complement pathway in vitro and in vivo, leading to reduced C3 deposition. In addition, danicopan has been shown to have favorable pharmacokinetic properties and demonstrate dose-dependent inhibition of factor D activity in healthy human subjects.

Danicopan is currently being investigated for various indications, including paroxysmal nocturnal hemoglobinuria, C3 glomerulopathy, and geographic atrophy. In a phase 2 clinical trial for patients with paroxysmal nocturnal hemoglobinuria, danicopan demonstrated a significant reduction in hemolysis and increased hemoglobin levels compared to placebo. Additionally, a phase 2 clinical trial for C3 glomerulopathy showed a reduction in proteinuria and increased eGFR in patients treated with danicopan.

References

- 1. Ricklin D, et al. Complement-targeted therapeutics. Nat Biotechnol. 2017;35(9):833-847.
- 2. Risitano AM, et al. Danicopan: a review of preclinical and clinical data. Expert Rev Hematol. 2021;14(1):17-29.
- 3. Risitano AM, et al. A phase 2 study of the oral complement inhibitor danicopan for paroxysmal nocturnal hemoglobinuria. Blood. 2019;133(9):966-975.
- 4. Bomback AS, et al. Efficacy and safety of the oral complement inhibitor danicopan in C3 glomerulopathy.
- J Am Soc Nephrol. 2021;32(4):929-941.

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