

## Product Data Sheet

Product Name: PTC299  
Cat. No.: GC65335

### Chemical Properties

Cas. No. 1256565-36-2

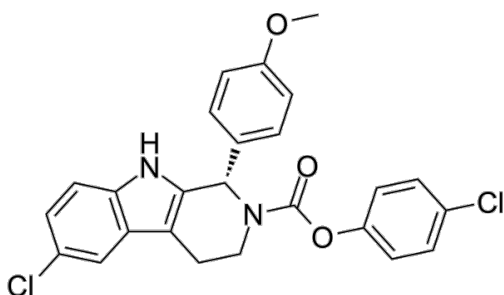
Formula C<sub>25</sub>H<sub>20</sub>Cl<sub>2</sub>N<sub>2</sub>O<sub>3</sub> M.Wt 467.34

Solubility DMSO : 50 mg/mL (106.99 mM; Need ultrasonic) Storage Store at -20°C

General tips For obtaining a higher solubility , please warm the tube at 37 °C and shake it in the ultrasonic bath for a while. Stock solution can be stored below -20°C for several months.

Shipping Condition Evaluation sample solution : ship with blue ice All other available size: ship with RT , or blue ice upon request.

Structure



### Background

PTC299 is an orally active inhibitor of VEGFA mRNA translation that selectively inhibits VEGF protein synthesis at the post-transcriptional level. PTC299 is also a potent inhibitor of dihydroorotate

**Caution: Product has not been fully validated for medical applications. For research use only.**

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dehydrogenase (DHODH). PTC299 shows good oral bioavailability and lack of off-target kinase inhibition and myelosuppression. PTC299 can be useful for the research of hematologic malignancies[1][2].

PTC299 inhibits hypoxia-induced VEGFA protein production in HeLa cells with an EC50 of  $1.64 \pm 0.83$  nM[1]. PTC299 is the most potent inhibitor with an IC50 of about 1 nM, over 10 to 1000-fold more potent than Brequinar, Vidofludimus or A 77-1726 in leukemia cells[1].

[1]. Cao L, et al. Targeting of Hematologic Malignancies with PTC299, A Novel Potent Inhibitor of Dihydroorotate Dehydrogenase with Favorable Pharmaceutical Properties. Mol Cancer Ther. 2019 Jan;18(1):3-16.

[2]. Bender Ignacio RA, et al. Brief Report: A Phase 1b/Pharmacokinetic Trial of PTC299, a Novel PostTranscriptional VEGF Inhibitor, for AIDS-Related Kaposi's Sarcoma: AIDS Malignancy Consortium Trial 059. J Acquir Immune Defic Syndr. 2016 May 1;72(1):52-7.

[3]. Packer RJ, et al. Phase I and pharmacokinetic trial of PTC299 in pediatric patients with refractory or recurrent central nervous system tumors: a PBTC study. J Neurooncol. 2015;121(1):217-224.

[4]. Zeng Z, et al, Targeting dihydroorotate dehydrogenase in acute myeloid leukemia. Haematologica. 2018 Sep;103(9):1415-1417.

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