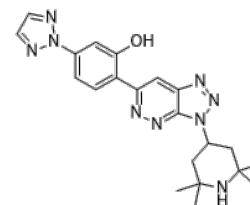


Product Name : Votoplam
Cat. No. : PC-23055
CAS No. : 2407849-89-0
Molecular Formula : C₂₁H₂₅N₉O
Molecular Weight : 419.49
Target : Spliceosome
Solubility : 10 mM in DMSO



CAS: 2407849-89-0

Biological Activity

Votoplam (PTC518) is a small molecule **HTT gene modulator** and splicing modifier, selectively lowers **huntingtin mRNA** and protein.

Votoplam (PTC518) promotes the inclusion of a novel pseudoexon containing a premature termination codon, thus triggering HTT mRNA degradation and subsequent reduction in HTT protein levels.

Votoplam (PTC518) demonstrated a dose-dependent lowering of HTT mRNA and protein to the targeted 30-50% reduction in a Phase 1 healthy volunteer study of PTC518 for Huntington's disease.

References

Patent WO2020005873 A1.

Caution: Product has not been fully validated for medical applications. Lab Use Only!

E-mail: tech@probechem.com