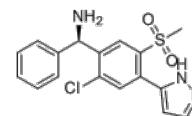


Product Name : NVS1.1
Cat. No. : PC-21294
CAS No. :
Molecular Formula : C₁₇H₁₆ClN₃O₂S
Molecular Weight : 361.84
Target : Other Targets
Solubility : 10 mM in DMSO



Biological Activity

NVS1.1 is a potent readthrough promoter, promotes the rapid and specific degradation of eukaryotic release factor 1 (**eRF1**) and restores functional full-length proteins in cystic fibrosis and Hurler disease models.

NVS1.1 restores α -L-iduronidase (IDUA) enzyme activity and reduces the GAG levels in a dose-dependent manner in human patient-derived fibroblasts.

NVS1.1 stimulates premature termination codon (PTC) suppression by triggering rapid proteasomal degradation of the translation termination factor eRF1.

NVS1.1 blocks translation termination by trapping eRF1 in the A site, causing ribosome collisions.

NVS1.1 is efficacious in a rat Hurler IDUA-W401X animal model, shows good drug-like properties with favorable pharmacokinetics, metabolic stability, and suitability for oral administration.

References

Gurzeler LA, et al. Cell Rep. 2023 Sep 26;42(9):113056.

Patent WO2014/091446A1

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

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