

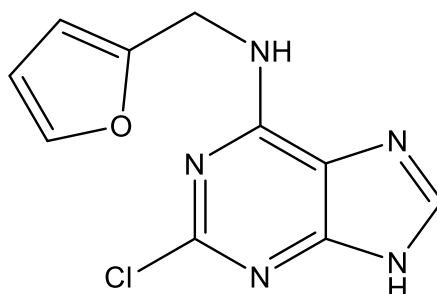
Catalog # 10-5273

RECTAS

CAS# 101862-47-9

2-Chloro-N-(2-furanylmethyl)-9H-purin-6-amine

Lot # E103885



RECTAS, a pre-mRNA splice modulator (Rectifier of Aberrant Splicing), was discovered in a small molecule screen for compounds that rectify aberrant IKBKAP splicing in cells from patients with familial dysautonomia. RECTAS (2 μ M) increased the expression of IKAP and restored tRNA modifications.^{1,2} It directly interacts with CDC-like kinases enhancing SRSF6 phosphorylation.² A combination treatment with RECTAS and phenylbutyric acid was shown to restore DJ-1 protein and mitochondrial dysfunction in Parkinson's disease models.³

- 1) Yoshida *et al.* (2015), *Rectifier of aberrant mRNA splicing recovers tRNA modification in familial dysautonomia*; Proc. Natl. Acad. Sci. USA **112** 2764
- 2) Ajiro *et al.* (2021), *Therapeutic manipulation of IKBKAP mis-splicing with a small molecule to cure familial dysautonomia*; Nat. Commun. **12** 4507
- 3) Boussaad *et al.* (2020), *A patient-based model of RNA mis-splicing uncovers treatment targets in Parkinson's disease*; Sci. Transl. Med. **12** eaau3960

PHYSICAL DATA

Molecular Weight:	249.66
Molecular Formula:	C ₁₀ H ₈ ClN ₅ O
Purity:	>98% by TLC
	NMR: (Conforms)
Solubility:	DMSO (27 mg/ml)
Physical Description:	Pale yellow solid
Storage and Stability:	Store as supplied at -20C for up to 2 years from the date of purchase. Solutions in DMSO may be stored at -20°C for up to 3 months.

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