

Product Name: RECTAS

Catalog No.: 7679

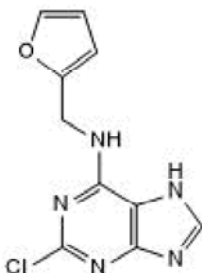
Batch No.: 1

CAS Number: 101862-47-9

IUPAC Name: 2-Chloro-*N*-(2-furanylmethyl)-7*H*-purin-6-amine

1. PHYSICAL AND CHEMICAL PROPERTIES

Batch Molecular Formula: C₁₀H₈ClN₅O.
Batch Molecular Weight: 249.66
Physical Appearance: Off White solid
Solubility: DMSO to 100 mM
Storage: Store at -20°C
Batch Molecular Structure:



2. ANALYTICAL DATA

HPLC: Shows 97.6% purity
¹H NMR: Consistent with structure
Mass Spectrum: Consistent with structure

Microanalysis:

	Carbon	Hydrogen	Nitrogen
Theoretical	48.11	3.23	28.05
Found	47.91	3.21	28.32

Caution - Not Fully Tested • Research Use Only • Not For Human or Veterinary Use

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Description:

RECTAS (rectifier of aberrant splicing) is a pre-mRNA splicing modulator of the IKBKAP (inhibitor of κ light polypeptide gene enhancer in B-cells, kinase complex associated protein) gene. It corrects aberrant IKBKAP splicing in vitro in cellular models of familial dysautonomia and in vivo in transgenic mice. In combination with Phenylbutyric acid (Cat. No. 2682), RECTAS rescues PARK7 aberrant splicing and neuronal cell loss in a midbrain organoid model of familial Parkinson's disease. Orally bioavailable and brain penetrant.

Physical and Chemical Properties:

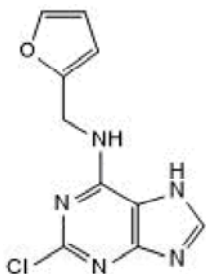
Batch Molecular Formula: C₁₀H₈ClN₅O.

Batch Molecular Weight: 249.66

Physical Appearance: Off White solid

Minimum Purity: ≥98%

Batch Molecular Structure:



Storage: Store at -20°C

Solubility & Usage Info:

DMSO to 100 mM

Stability and Solubility Advice:

Some solutions can be difficult to obtain and can be encouraged by rapid stirring, sonication or gentle warming (in a 45-60°C water bath).

Information concerning product stability, particularly in solution, has rarely been reported and in most cases we can only offer a general guide. Our standard recommendations are:

SOLIDS: Provided storage is as stated on the product label and the vial is kept tightly sealed, the product can be stored for up to 6 months from date of receipt.

SOLUTIONS: We recommend that stock solutions, once prepared, are stored aliquoted in tightly sealed vials at -20°C or below and used within 1 month. Wherever possible solutions should be made up and used on the same day.

References:

Ajiro et al (2021) Therapeutic manipulation of IKBKAP mis-splicing with a small molecule to cure familial dysautonomia. *Nat. Commun.* **12** 4507. PMID: 34301951 .

Boussaad et al (2020) A patient-based model of RNA mis-splicing uncovers treatment targets in Parkinson's disease. *Sci. Transl. Med.* **12** eaau3960. PMID: 32908004.

Yoshida et al (2015) Rectifier of aberrant mRNA splicing recovers tRNA modification in familial dysautonomia. *Proc. Natl. Acad. Sci U.S.A.* **112** 2764. PMID: 25675486.

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bio-techne.com

info@bio-techne.com

techsupport@bio-techne.com

North America

Tel: (800) 343 7475

China

info.cn@bio-techne.com

Tel: +86 (21) 52380373

Europe Middle East Africa

Tel: +44 (0)1235 529449

Rest of World

www.tocris.com/distributors

Tel: +1 612 379 2956