

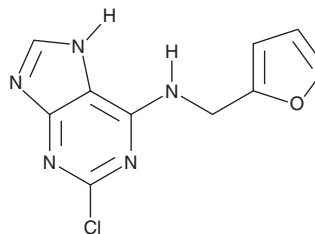
# PRODUCT INFORMATION



## RECTAS

Item No. 38322

**CAS Registry No.:** 101862-47-9  
**Formal Name:** 2-chloro-N-(2-furanylmethyl)-9H-purin-6-amine  
**Synonyms:** NSC 45794, Rectifier of Aberrant Splicing  
**MF:** C<sub>10</sub>H<sub>8</sub>ClN<sub>5</sub>O  
**FW:** 249.7  
**Purity:** ≥98%  
**Supplied as:** A solid  
**Storage:** -20°C  
**Stability:** ≥4 years



Information represents the product specifications. Batch specific analytical results are provided on each certificate of analysis.

### Laboratory Procedures

RECTAS is supplied as a solid. A stock solution may be made by dissolving the RECTAS in the solvent of choice, which should be purged with an inert gas. RECTAS is soluble in organic solvents such as DMSO and dimethyl formamide. The solubility of RECTAS in these solvents is approximately 20 mg/ml. It is slightly soluble in ethanol.

Further dilutions of the stock solution into aqueous buffers or isotonic saline should be made prior to performing biological experiments. Ensure that the residual amount of organic solvent is insignificant, since organic solvents may have physiological effects at low concentrations. Organic solvent-free aqueous solutions of RECTAS can be prepared by directly dissolving the solid powder in aqueous buffers. RECTAS is slightly soluble in PBS (pH 7.2). We do not recommend storing the aqueous solution for more than one day.

### Description

RECTAS is a pre-mRNA splicing modulator.<sup>1-4</sup> It corrects aberrant splicing in a splicing reporter assay for disease genes with dual color (SPREADD) using HeLa cells expressing *IKBKAP*, the gene encoding elongator complex protein 1 (ELP1), with a point mutation in intron 20 (IVS20 + 6T>C), which is associated with the neurodegenerative disease familial dysautonomia (FD).<sup>1</sup> RECTAS (2 μM) restores the modification of cytoplasmic tRNAs with uridine in the wobble position in primary human fibroblasts from patients with FD to the same level as in primary human fibroblasts from carriers of the FD mutation. It increases the mRNA levels of correctly spliced *PARK7*, a gene whose mutations are associated with early-onset Parkinson's disease, in neural precursor cells differentiated from fibroblasts of patients with the homozygous *PARK7* c.192G>C exonic splicing mutation when used in combination with the chemical chaperone phenylbutyric acid (Item No. 11323).<sup>3</sup> Intratumoral administration of RECTAS decreases tumor volume and increases survival and the number of tumor-infiltrating CD8<sup>+</sup> T cells in an MC-38 murine colon carcinoma model.<sup>4</sup>

### References

1. Yoshida, M., Kataoka, N., Miyauchi, K., *et al.* Rectifier of aberrant mRNA splicing recovers tRNA modification in familial dysautonomia. *Proc. Natl. Acad. Sci. USA*. **112(9)**, 2764-2769 (2015).
2. Ajiro, M., Awaya, T., Kim, Y.J., *et al.* Therapeutic manipulation of *IKBKAP* mis-splicing with a small molecule to cure familial dysautonomia. *Nat. Commun.* **12(10)**, 4507 (2021).
3. Boussaad, I., Obermaier, C.D., Hanss, Z., *et al.* A patient-based model of RNA mis-splicing uncovers treatment targets in Parkinson's disease. *Sci. Transl. Med.* **12(560)**, eaau3960 (2020).
4. Matsushima, S., Ajiro, M., Iida, K., *et al.* Chemical induction of splice-neoantigens attenuates tumor growth in a preclinical model of colorectal cancer. *Sci. Transl. Med.* **14(673)**, eabn6056 (2022).

#### WARNING

THIS PRODUCT IS FOR RESEARCH ONLY - NOT FOR HUMAN OR VETERINARY DIAGNOSTIC OR THERAPEUTIC USE.

#### SAFETY DATA

This material should be considered hazardous until further information becomes available. Do not ingest, inhale, get in eyes, on skin, or on clothing. Wash thoroughly after handling. Before use, the user must review the [complete](#) Safety Data Sheet, which has been sent via email to your institution.

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