

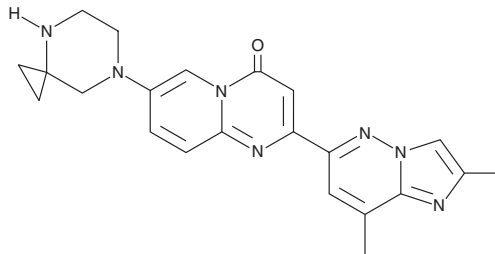
PRODUCT INFORMATION



Risdiplam

Item No. 29028

CAS Registry No.: 1825352-65-5
Formal Name: 7-(4,7-diazaspiro[2.5]oct-7-yl)-2-(2,8-dimethylimidazo[1,2-b]pyridazin-6-yl)-4H-pyrido[1,2-a]pyrimidin-4-one
Synonyms: RG-7916, RO7034067
MF: C₂₂H₂₃N₇O
FW: 401.5
Purity: ≥98%
UV/Vis.: λ_{max}: 255, 287, 404 nm
Supplied as: A crystalline 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

Risdiplam is supplied as a crystalline solid. A stock solution may be made by dissolving the risdiplam in the solvent of choice, which should be purged with an inert gas. Risdiplam is soluble in the organic solvent chloroform at a concentration of approximately 10 mg/ml.

Description

Risdiplam is a survival of motor neuron-2 (SMN2) splicing modifier.¹ It enhances SMN2 splicing (EC_{1.5x} = 40 nM) and increases SMN protein levels (EC_{1.5x} = 163 nM) in cellular assays. Risdiplam (3 mg/kg) increases brain and quadricep muscle SMN protein levels in the adult C/C-allele mouse model of mild spinal muscular atrophy (SMA) and the SMND7 neonate mouse model of severe SMA. It also increases the number of vesicular glutamate transporter 1 (vGLUT1) proprioceptive inputs on motor neurons, the number of L3 to L5 spinal motor neurons, the percentage of fully innervated neuromuscular junctions (NMJs), and the size of the extensor digitorum longus muscles in adult SMND7 mice. Formulations containing risdiplam have been used in the treatment of spinal muscular atrophy.

Reference

1. Ratni, H., Ebeling, M., Baird, J., *et al.* Discovery of risdiplam, a selective survival of motor neuron-2 (SMN2) gene splicing modifier for the treatment of spinal muscular atrophy (SMA). *J. Med. Chem.* **61**(15), 6501-6517 (2018).

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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