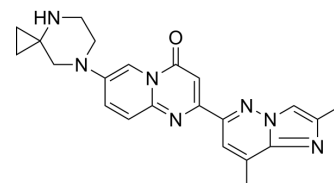


## Risdiplam

Cat. No.:	HY-109101		
CAS No.:	1825352-65-5		
Molecular Formula:	C <sub>22</sub> H <sub>23</sub> N <sub>7</sub> O		
Molecular Weight:	401.46		
Target:	DNA/RNA Synthesis		
Pathway:	Cell Cycle/DNA Damage		
Storage:	Powder	-20°C	3 years
		4°C	2 years
	In solvent	-80°C	6 months
		-20°C	1 month



### SOLVENT & SOLUBILITY

#### In Vitro

DMSO : 1.96 mg/mL (4.88 mM; ultrasonic and warming and adjust pH to 5 with HCl and heat to 60°C)  
 Ethanol : < 1 mg/mL (insoluble)  
 H<sub>2</sub>O : < 0.1 mg/mL (insoluble)

Preparing Stock Solutions	Solvent Concentration	Mass		
		1 mg	5 mg	10 mg
	1 mM	2.4909 mL	12.4545 mL	24.9091 mL
	5 mM	---	---	---
	10 mM	---	---	---

Please refer to the solubility information to select the appropriate solvent.

### BIOLOGICAL ACTIVITY

#### Description

Risdiplam (RG7916) is an orally administered, centrally and peripherally distributed *SMN2* pre-mRNA splicing modifier that increases survival motor neuron (SMN) protein levels<sup>[1]</sup>.

#### IC<sub>50</sub> & Target

SMN2

#### In Vitro

Risdiplam modulates *SMN2* pre-mRNA splicing towards the production of full-length *SMN2* mRNA and increases SMN protein levels. Risdiplam is a modifier of *SMN2* splicing, leading to an increase in *SMN2* full length transcript and thus functional SMN protein. Spinal muscular atrophy (SMA) type I remains the most common genetic disease resulting in death in infancy. Characterized by progressive motor and respiratory muscle weakness, this autosomal recessive neuromuscular disorder is caused by low levels of the survival motor neuron protein (SMN) due to inactivating bi-allelic deletions and other disabling mutations in the survival motor neuron 1 (*SMN1*) gene<sup>[1]</sup>.  
 MCE has not independently confirmed the accuracy of these methods. They are for reference only.

---

## CUSTOMER VALIDATION

- Nat Commun. 2023 Jun 10;14(1):3435.
- Nucleic Acids Res. 2023 Apr 7;gkad259.
- Nucleic Acids Res. 2021 Sep 7;49(15):8462-8470.
- Cell Mol Life Sci. 2022 Jul 22;79(8):441.
- bioRxiv. 2023 Jul 27.

See more customer validations on [www.MedChemExpress.com](http://www.MedChemExpress.com)

---

## REFERENCES

[1]. Poirier A, et al. Risdiplam distributes and increases SMN protein in both the central nervous system and peripheral organs. Pharmacol Res Perspect. 2018 Nov 29;6(6):e00447.

---

**Caution: Product has not been fully validated for medical applications. For research use only.**

Tel: 609-228-6898

Fax: 609-228-5909

E-mail: [tech@MedChemExpress.com](mailto:tech@MedChemExpress.com)

Address: 1 Deer Park Dr, Suite Q, Monmouth Junction, NJ 08852, USA