

**Product Name:** Risdiplam

**Catalog No.:** 7916

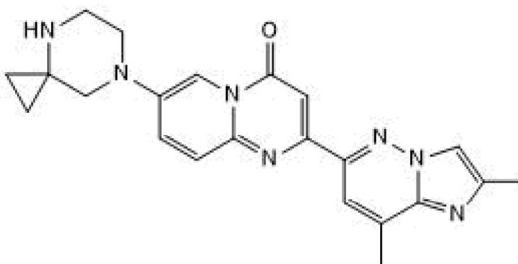
**Batch No.:** 1

CAS Number: 1825352-65-5

IUPAC 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

**1. PHYSICAL AND CHEMICAL PROPERTIES**

**Batch Molecular Formula:** C<sub>22</sub>H<sub>23</sub>N<sub>7</sub>O  
**Batch Molecular Weight:** 401.47  
**Physical Appearance:** Yellow solid  
**Solubility:** 1eq. HCl to 100 mM with gentle warming  
**Storage:** Store at -20°C  
**Batch Molecular Structure:**



**2. ANALYTICAL DATA**

**HPLC:** Shows 99.9% purity  
**<sup>1</sup>H NMR:** Consistent with structure  
**Mass Spectrum:** Consistent with structure

**Microanalysis:**

	Carbon	Hydrogen	Nitrogen
Theoretical	65.82	5.77	24.42
Found	66.07	5.76	24.31

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

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**Description:**

Risdiplam is a selective survival of motor neuron-2 (SMN2) pre-mRNA splicing modifier. It potently enhances SMN2 splicing in vitro ( $EC_{1.5X} = 4$  nM) and increases SMN protein in both brain (up to 100%) and muscle tissues (up to 49%) of transgenic mouse models of spinal muscular atrophy (SMA). Risdiplam dose-dependently increases the number of vesicular glutamate transporter 1 (vGlut1) proprioceptive inputs on motor neurons, the number of L3 to L5 motor neurons, the percentage of fully innervated neuromuscular junctions, and muscle size in the extensor digitorum longus muscles in SMN  $\Delta 7$  mice.

**Physical and Chemical Properties:**

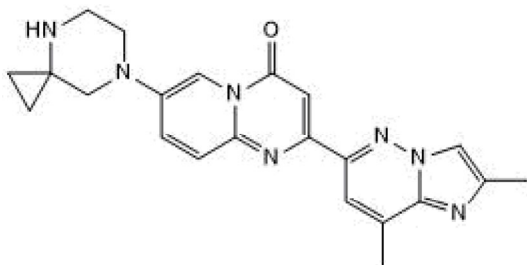
Batch Molecular Formula:  $C_{22}H_{23}N_7O$

Batch Molecular Weight: 401.47

Physical Appearance: Yellow solid

**Minimum Purity:**  $\geq 98\%$

**Batch Molecular Structure:**



**References:**

**Markati et al (2022)** Risdiplam: an investigational survival motor neuron 2 (SMN2) splicing modifier for spinal muscular atrophy (SMA). *Expert Opin. Investig. Drugs* **31** 451. PMID: 35316106.

**Paik et al (2022)** Risdiplam: A Review in Spinal Muscular Atrophy. *CNS Drugs* **36** 401. PMID: 35284988.

**Martin et al (2021)** Screening strategies for identifying RNA- and ribonucleoprotein-targeted compounds. *Trends Pharmacol. Sci.* **42** 758. PMID: 34215444.

**Storage:** Store at  $-20^{\circ}C$

**Solubility & Usage Info:**

1eq. HCl to 100 mM with gentle warming

**Stability and Solubility Advice:**

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

Information concerning product stability, particularly in solution, has rarely been reported and in most cases we can only offer a general guide. \*Unless contradicted by product-specific protocols or instructions, our standard recommendations apply:

**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  $-20^{\circ}C$  or below and used within 1 month. Wherever possible solutions should be made up and used on the same day.

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