

## **Product** Data Sheet

## mHTT-IN-2

Pathway:

 Cat. No.:
 HY-156103 

 CAS No.:
 2743336-70-9 

 Molecular Formula:
  $C_{20}H_{22}FN_7O$  

 Molecular Weight:
 395.43 

Target: Huntingtin

Storage: Please store the product under the recommended conditions in the Certificate of

Analysis.

**Neuronal Signaling** 

HN N N H

## **BIOLOGICAL ACTIVITY**

Description

mHTT-IN-2 (compound 27) is a potent inhibitor (EC<sub>50</sub>=0.066 μM) of mutant huntingtin (mHTT). mHTT-IN-2 reduces canonical splicing of HTT RNA exons [49-50] and is a splicing regulator of the huntingtin (HTT) gene. mHTT-IN-2 exhibits inhibitory activity in vitro and in vivo in human HD stem cells and mouse BACHD models. mHTT-IN-2 may be used in the study of branaplam-related peripheral neuropathy<sup>[1]</sup>.

IC<sub>50</sub> & Target

EC50: 0.066 μM (mHTT protein)<sup>[1]</sup>

## **REFERENCES**

[1]. Liu L, et al. Identification and Optimization of RNA-Splicing Modulators as Huntingtin Protein-Lowering Agents for the Treatment of Huntington's Disease. J Med Chem. 2023 Sep 15...

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

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Inhibitors