

Product Data Sheet

Product Name: Nusinersen

Cat. No.: GC64293

Chemical Properties

Cas. No. 1258984-36-9

Formula $C_{234}H_{340}N_{61}O_{128}P_{17}S_{17}$ M.Wt 7127.30

Solubility Storage -20°C, stored under nitrogen, away from moisture

General tips For obtaining a higher solubility, please warm the tube at 37 °C and shake it in the ultrasonic bath for a while. Stock solution can be stored below -20°C for several months.

Shipping Condition Evaluation sample solution: ship with blue ice. All other available size: ship with RT, or blue ice upon request.

Structure
 RNA, [2'-O-(2-methoxyethyl)](P-thio)
 (m5U-m5C-A-m5C-m5U-m5U-m5U-
 m5C-A-m5U-A-A-m5U-G-m5C-m5U-
 G-G)

Protocol

Animal experiment [1]:

Animal models WT mice and SMNΔ7 mice

Preparation Method To investigate the effects of nusinersen treatment in the αMNs of SMNΔ7 mice, we used 32 animals (16 WT mice and 16 SMNΔ7 mice) that were divided into four groups. WT and SMNΔ7 mice were treated with 10μl of nusinersen at a dose of 6.5mg/kg (nusinersen group), or with 10μl of a 0.9% saline solution (vehicle group). nusinersen or the vehicle was injected at the day of birth (P0) via ICV using a 10-μl syringe and a custom 33-gauge needle.

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

Tel: (909) 407-4943 Fax: (626) 353-8530 E-mail: tech@glpbio.com

Address: 10292 Central Ave. #205, Montclair, CA, USA

Product Data Sheet

Dosage form 6.5mg/kg; I.C.V.

Applications Treatment with nusinersen robustly rescues the growth curve, enhances motor function and dramatically reduces α MN loss.

References:

[1] Berciano M T, Puente-Bedia A, Medina-Samamé A, et al. Nusinersen ameliorates motor function and prevents motoneuron Cajal body disassembly and abnormal poly (A) RNA distribution in a SMA mouse model[J]. Scientific reports, 2020, 10(1): 10738.

Background

Nusinersen is an SMN-ASO that increases SMN levels by blocking an intronic splice silencer in SMN2, thereby facilitating the exon 7 inclusion and generation of FL-SMN2 transcripts^[1].

Nusinersen(6.5mg/kg; I.C.V.) robustly rescues the growth curve of SMN Δ 7 mice, enhances motor function and dramatically reduces α MN loss and prevents the disruption and loss of canonical Cajal bodies (CBs) in α MNs of the SMA mouse. Also, Nusinersen treatment normalizes the distribution of the protein synthesis machinery and polyadenylated mRNAs in SMN Δ 7 mice^[2]. Nusinersen was administered to rabbits as an intrathecally (IT) bolus dose of 1.8 mg/kg to the first group, intravenously (IV) bolus dose of 1 mg/kg to the second group, respectively. The C_{max} , AUC_{last} and C_0 of IV were $15.6 \pm 4.88 \mu\text{g/mL}$, $17.8 \pm 4.10 \text{ h} \cdot \mu\text{g/mL}$ and $17.6 \pm 6.20 \mu\text{g/mL}$; the T_{max} , C_{max} and AUC_{last} of IT were 1h, $6.0 \pm 0.10 \mu\text{g/mL}$ and $12.4 \pm 1.15 \text{ h} \cdot \mu\text{g/mL}$, respectively. The calculated bioavailability of nusinersen was 33 %^[3].

References:

[1] Torres-Benito L, Schneider S, Rombo R, et al. NCALD antisense oligonucleotide therapy in addition to nusinersen further ameliorates spinal muscular atrophy in mice[J]. The American Journal of Human Genetics, 2019, 105(1): 221-230.

[2] Berciano M T, Puente-Bedia A, Medina-Samamé A, et al. Nusinersen ameliorates motor function and prevents motoneuron Cajal body disassembly and abnormal poly (A) RNA distribution in a SMA mouse model[J]. Scientific reports, 2020, 10(1): 10738.

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

Tel: (909) 407-4943 Fax: (626) 353-8530 E-mail: tech@glpbio.com

Address: 10292 Central Ave. #205, Montclair, CA, USA

Product Data Sheet

[3] Zhang X, Sha C, Zhang W, et al. Development and validation of an HILIC/MS/MS method for determination of nusinersen in rabbit plasma[J]. Heliyon, 2024, 10(10).

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

Tel: (909) 407-4943 Fax: (626) 353-8530 E-mail: tech@glpbio.com

Address: 10292 Central Ave. #205, Montclair, CA, USA