

Product Data Sheet

Product Name: Golodirsen

Cat. No.: GC64458

Chemical Properties

Cas. No. 1422959-91-8

Formula M.Wt 8647

Solubility H₂O : 100 mg/mL (11.56 mM; Need ultrasonic) Storage Store at -20°C

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

Golodirsen

Background

Golodirsen (SRP-4053) is a phosphorodiamidate morpholino oligomer (PMO) that specifically targets exon 53 of dystrophin pre-mRNA. Golodirsen can be used for the research of Duchenne muscular dystrophy (DMD)[1][2].

Golodirsen specifically targets exon 53 of dystrophin pre-mRNA, resulting in its exclusion from the final mRNA product. Skipping of exon 53 results in restoration of the mRNA reading frame and leads to the production of a partially internally deleted dystrophin protein with intact C and N-terminal

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

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regions[2].

[1]. Heo YA. Golodirsén: First Approval. *Drugs*. 2020 Feb;80(3):329-333.

[2]. Scaglioni D, et, al. The administration of antisense oligonucleotide golodirsén reduces pathological regeneration in patients with Duchenne muscular dystrophy. *Acta Neuropathol Commun*. 2021 Jan 6;9(1):7.

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