

Product Data Sheet

Product Name: Viltolarsen

Cat. No.: GC63876

Chemical Properties

Cas. No. 2055732-84-6

Formula

M.Wt

Solubility H₂O : 100 mg/mL (14.44 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

Viltolarsen

Background

Viltolarsen (NS-065/NCNP-01), a phosphorodiamidate morpholino antisense oligonucleotide, targets the splicing of exon 53 in the dystrophin gene. Viltolarsen can be used for the research of the Duchenne muscular dystrophy (DMD)[1].

Duchenne muscular dystrophy (DMD) is one of the most common lethal muscle-wasting disorders affecting young boys caused by mutations in the DMD gene. Exon skipping has emerged as a promising therapy for DMD. Antisense oligonucleotides (AONs) are designed to induce the skipping

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

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of exon(s), in order to restore the reading frame, and therefore, allow for dystrophin expression[1].

[1]. Dzierlega K, et al. Optimization of antisense-mediated exon skipping for Duchenne muscular dystrophy. *Gene Ther.* 2020;27(9):407-416.

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