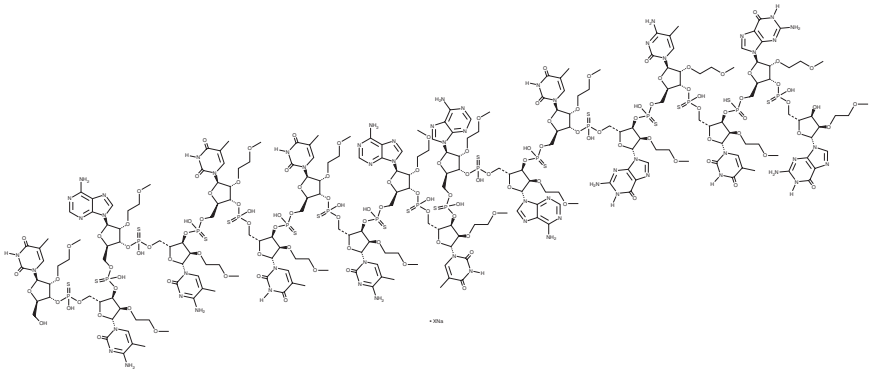
PRODUCT INFORMATION

Nusinersen

Item No. 46574



Formal name:	[2′-O-(2-methoxyethyl)](P-thio)(m^{5}U-m^{5}C-A-m^{5}C-m^{5}U-m^{5}U-m^{5}U-m^{5}C-A-m^{5}U-A-A-m^{5}U-G-m^{5}C-m^{5}U-G-G), RNA, sodium salt
Synonym:	ASO 10-27
MF:	$C_{234}H_{340}N_{61}O_{128}P_{17}S_{17}$ • XNa
FW:	7,127.2
Purity:	≥95%
Supplied as:	A solid
Storage:	-20°C
Stability:	≥4 years

Information represents the product specifications. Batch specific analytical results are provided on each certificate of analysis.

Laboratory Procedures

Nusinersen is supplied as a solid. A stock solution may be made by dissolving the nusinersen in the solvent of choice, which should be purged with an inert gas. Nusinersen is sparingly soluble (1-10 mg/ml) in a 1:1 solution of acetonitrile:water.

Description

Nusinersen is an antisense oligonucleotide (ASO) that is 18 nucleotides in length and contains a phosphorothioate backbone and 2'-O-methoxyethyl (MOE) nucleotides.[1] Intracerebroventricular administration of nusinersen increases cortical and thoracic spinal cord levels of full-length human survival motor neuron protein (SMN) in an *Smn*$^{+/-}$;*SMN2*$^{+/+}$ mouse model of spinal muscular atrophy (SMA). Formulations containing nusinersen have been used in the treatment of SMA.

Reference

1. Hua, Y., Sahashi, K., Hung, G., *et al*. Antisense correction of SMN2 splicing in the CNS rescues necrosis in a type III SMA mouse model. *Genes Dev.* **24(15)**, 1634-16344 (2010).

WARNING
THIS PRODUCT IS FOR RESEARCH ONLY - NOT FOR HUMAN OR VETERINARY DIAGNOSTIC OR THERAPEUTIC USE.

SAFETY DATA
This material should be considered hazardous until further information becomes available. Do not ingest, inhale, get in eyes, on skin, or on clothing. Wash thoroughly after handling. Before use, the user must review the complete Safety Data Sheet, which has been sent *via* email to your institution.

WARRANTY AND LIMITATION OF REMEDY
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