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Galsulfase In Vitro Transcribed mRNA-LNP

Catalog Number:SG-MRNA-LNP-1907

DESCRIPTION	
Product Name	Galsulfase In Vitro Transcribed mRNA-LNP
Gene Name	Galsufase
Source	The ORF of Galsulfase was cloned in our IVT vector and mRNA was prepared through in vitro transcription and purification. The purified mRNA was further encapsulated with LNP(DSPC:Cholesterol:DMG-PEG:SM102).
Alternative names	Galsulfase
SPECIFICATIONS	
Cap	m7GpppN
5'-UTR	5' -untranslated region derived from human alpha-globin RNA with an optimized Kozak sequence
ORF	Galsulfase
3'-UTR	3' UTR comprising two sequence elements derived from the aminoterminal enhancer of split (AES) mRNA and the mitochondrial encoded 12S ribosomal RNA
Poly(A) Tail	A 110-nucleotide poly(A)-tail consisting of a stretch of 30 adenosine residues, followed by a 10-nucleotide linker sequence and another 70 adenosine residues.
Modifications	N1-methyl-pseudouridine
Neutral Lipid	1,2-distearoyl-sn-glycero-3-phosphocholine (DSPC)
Cholesterol	Cholesterol
Lonizable Lipid	1,2-dimyristoyl-rac-glycero-3-methoxypolyethylene glycol-2000 (PEG2000-DMG)
PEG-lipid	Heptadecan-9-yl 8-((2-hydroxyethyl)(8-(nonyloxy)−8-oxooctyl)amino)octanoate)(SM-102)
Storage	-20 °C
Buffer	PBS, pH7.4
Cryoprotectant	Trehalose
BACKGROUND	
Gene Accession	
Gene Alias	Galsulfase

	asparagine-linked glycosylation sites, four of which carry a bis mannose-6-phosphate manose7 oligosaccharide for specific cellular recognition. Post-translational modification of Cys53 produces the catalytic amino acid residue Ca-formylglycine, which is required for enzyme activity and is conserved in all members of the sulfatase enzyme family. Galsulfase supplies recombinant-engineered galsulfase, a normal variant form of the polymorphic human enzyme, N-acetylgalactosamine 4-sulfatase. It is a lysosomal hydrolase that catalyzes the cleavage of the sulfate ester from terminal N-acetylgalactosamine 4-sulfate residues of GAG chondroitin 4-sulfate and dermatan sulfate. Increased catabolism of GAG in turn reduces systemic dermatan sulfate accumulation, thereby reducing the primary symptoms of MPS VI.
Background	Galsufase is a variant form of the polymorphic human enzyme N-acetylgalactosamine 4-sulfatase of recombinant DNA origin. Galsulfase is a glycoprotein with a molecular weight of approximately 56 kD. The recombinant protein is comprised of 495 amino acids and contains six