

Alglucosidase- α In Vitro Transcribed mRNA-LNP

Catalog Number:SG-MRNA-LNP-1904

DESCRIPTION	
Product Name	Alglucosidase- α In Vitro Transcribed mRNA-LNP
Gene Name	GAA
Source	The ORF of Alglucosidase- α 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	Alglucosidase- α
SPECIFICATIONS	
Cap	m7GpppN
5'-UTR	5' -untranslated region derived from human alpha-globin RNA with an optimized Kozak sequence
ORF	Alglucosidase- α
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
Ionizable 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	Alglucosidase- α

glycogen by catalyzing the hydrolysis of α -1,4- and α -1,6- glycosidic linkages of lysosomal glycogen. Structurally, Alglucosidase alfa is a glycoprotein with a calculated mass of 98,008 daltons for the 883 residue mature polypeptide chain, and a total mass of approximately 109,000 daltons, including carbohydrates.

Background

Alglucosidase alfa consists of the human enzyme acid alpha-glucosidase (GAA) which is essential for the degradation of glycogen to glucose in lysosomes. It is encoded by the most predominant of nine observed haplotypes of this gene. Alglucosidase alfa degrades