

Agalsidase- β In Vitro Transcribed mRNA-LNP

Catalog Number:SG-MRNA-LNP-1908

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

prevents or reduces the severity of manifestations of Fabry disease such as renal failure, cardiomyopathy, or cerebrovascular events.

Background

α -galactosidase A is uptaken by cells via the mannose 6 phosphate receptor. Agalsidase beta hydrolyzes globotriaosylceramide and other glycosphingolipids that would normally be hydrolyzed by endogenous α -galactosidase A. Preventing the accumulation of glycosphingolipids