

DC82001 | 4A3-SC8

Instructions for Use

4A3-SC8 is a novel Ionizable amino lipid for RNA delivery. The CRISPR-Cas9 gene editing system has been a hotspot in the field of gene therapy, especially the gene correction induced by homology-directed repair (HDR). However, its application has various obstacles, such as large molecular weight, poor stability, off-target risk, and the complexity of codeliver multiple genes. Farbiak et al. established a novel ionizable lipid library consisting of four distinct amine cores (3A3, 3A5, 4A1, 4A3) and nine peripheries with different alkyl chain lengths (SC5-SC14), and screened out a class of iLNPs with ability of encapsulating Cas9 mRNA, sgRNA and donor DNA simultaneously. The delivery efficiency (quantified by luciferase mRNA expression) and iLNPs toxicity were evaluated with three different cell lines (HEK293T, HeLa, and IGROV-1), indicating the formulation containing 4A3-SC8 was the best. 4A3-SC8 iLNPs successfully induced HDR in HEK293 cells by one-pot delivery of Cas9 mRNA, sgRNA, and the correct ssDNA template. Confocal microscopy imaging showed that a portion of blue fluorescence in cells was corrected to green fluorescence. Furthermore, the nucleic acid ratios of Cas9: sgRNA: donor DNA loading in iLNPs at a ratio of 2:1:3 could maximize the HDR efficiency with the editing efficiency up to 23%, which breaks through the current bottleneck of HDR efficiency of only 1-5%. This progress is undoubtedly an important advance in the gene therapy field to cure diseases caused by genetic mutations.

Product information

CAS No.	Not available
Chemical Name	4A3-SC8
Formula	C75H139N3O16S4
M.Wt	1,467.2
Purity	ELSD-HPLC>95%
Storage	Original product: up to 1 year under the storage conditions stated on the vial, kept tightly sealed. Stock solutions: aliquots at -20°C, generally up to one month.
Shipping condition	Ships from Shanghai. Pure lipid is stable during ice-pack transport.

Formulation & study context

Literature values apply to the cited study only. Administration routes describe research, not clinical instructions.

A complete product-specific formulation has not been verified. No standard ratio is substituted.

N/P ratio	Not reported in the verified record
Cargo	Not available in the verified record
Administration route	Not available in the verified record
Animal model	Not available in the verified record
Formulation process	Not available in the verified record

N/P is a nitrogen-to-phosphate molar ratio. Lipid:cargo mass ratio is a different quantity and must not be used as N/P.

Stock-solution preparation table

Calculated using product-page MW: 1467.2 g/mol. Volumes are final solution volumes.

Mass	1 mM	5 mM	10 mM
10 mg	6.816 mL	1.363 mL	0.682 mL
25 mg	17.039 mL	3.408 mL	1.704 mL
50 mg	34.079 mL	6.816 mL	3.408 mL
100 mg	68.157 mL	13.631 mL	6.816 mL
250 mg	170.393 mL	34.079 mL	17.039 mL

Volume (mL) = mass (mg) × 1000 / [MW (g/mol) × concentration (mM)]. Mathematical conversions only; not measured solubility. Confirm solvent compatibility and the exact material before preparation.

Handling & storage

Provided that storage conditions are as stated on the product vial and the vial is kept tightly sealed, the product can be stored for up to 1 year.

Wherever possible, prepare and use solutions on the same day. If stock solutions must be prepared in advance, store them as aliquots in tightly sealed vials at -20°C. Generally, these will be usable for up to one month.

Before use, and prior to opening the vial, allow the product to equilibrate to room temperature for at least 1 hour.

References

1 Theranostic dendrimer-based lipid nanoparticles containing PEGylated BODIPY dyes for tumor imaging and systemic mRNA delivery in vivo-Journal of Controlled Release by Hu Xionga, Shuai Liua, Tuo Weia, Qiang Chenga, Daniel J. Siegwartab|||

2 Preparation of selective organ-targeting (SORT) lipid nanoparticles (LNPs) using multiple technical methods for tissue-specific mRNA delivery--Nature Protocols volume 18, pages265–291 (2023)