

DC67546 | ALC-0307

Instructions for Use

ALC 0307 is an ionizable amino lipid developed by Acuitas Therapeutics, serving as the critical functional component in lipid nanoparticles (LNPs) for targeted therapeutic delivery. As the core cationic lipid in specific LNP formulations (e.g., k-abe for CPS1-Q335X correction), its key feature is pH-dependent chargeability: it remains neutral at physiological pH but becomes positively charged in acidic environments like endosomes. This property enables efficient encapsulation of nucleic acid payloads (>97% efficiency, e.g., base editor mRNA/gRNA complexes) and facilitates endosomal escape via membrane disruption post-cellular uptake. Its optimized structure promotes selective hepatocyte targeting by binding endogenous apolipoprotein E (ApoE), which subsequently interacts with LDL receptors on liver cells. Preclinical studies show rapid clearance (>99.5% plasma reduction in 14 days) and manageable transient toxicity (mild, reversible cytoplasmic vacuolation in hepatocytes, short-term ALT/AST elevation). LNPs containing ALC0307, alongside helper lipids (cholesterol, DSPC, and PEG-lipid ALC-0159), form stable ~73 nm particles with low polydispersity. This combination enables repeatable, liver-directed delivery of gene editing therapeutics with minimized off-target effects, underpinning its use in individualized in vivo gene correction therapies.

Product information

CAS No.	Not available
Chemical Name	ALC-0307
Formula	C55H110O4N2
M.Wt	863.5
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: 863.5 g/mol. Volumes are final solution volumes.

Mass	1 mM	5 mM	10 mM
10 mg	11.581 mL	2.316 mL	1.158 mL
25 mg	28.952 mL	5.790 mL	2.895 mL
50 mg	57.904 mL	11.581 mL	5.790 mL
100 mg	115.808 mL	23.162 mL	11.581 mL
250 mg	289.519 mL	57.904 mL	28.952 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

Musunuru K, Grandinette SA, Wang X, et al. Patient-specific in vivo gene editing to treat a rare genetic disease. N Engl J Med 2025;392:2235-43. DOI: 10.1056/NEJMoa2504747