Table 1. Overview of in vivo studies on LNP-mediated delivery for gene editing and mRNA therapies. Click to expand. Summary of key studies, including delivery routes, LNP formulations, target tissues, and main outcomes in skeletal muscle and other organs.

Parameter	Kenjo et al. (2021)	Mochida et al. (2026)	Chen et al. (2023)	Carpenter et al. (2025)	Gao et al. (2023)
Model	C57BL/6J; hEx45KI-mdx44 (DMD model)	hEx45KI-mdx44; CAG-Luc2 hDMDEx45 KI; CAG-Luc2 hEx45KI-mdx44.	C57BL/6; BALB/c; mTmG reporter mice (Gt(ROSA)26Sortm4(ACTB-tdTomato,-EGFP)Luo/J); B16F10 melanoma mice	Taiwanese SMA model ($Smn1^{-/-} SMN2^{Tg/-}$); Sun1/sfGFP reporter mice (B6;129-Gt(ROSA)26Sortm5(CAG-Sun1/sfGFP)Nat/J)	Ai9 CRE reporter mice (B6.Cg-Gt(ROSA)26Sortm9(CAG-tdTomato)Hze/J)
LNP Composition (molar ratio)	TCL053 DPPC Cholesterol PEG-DMG (60:10.6:28.7:0.7)	TCL053 DPPC Cholesterol PEG-DMG (60:10.6:28.7:0.7)	iso-A11B5C DOPE Cholesterol C14-PEG2000 (60:10:29:1)	SM102 DSPC Cholesterol DMG-PEG-2000(50:10:38.5:1.5)	D-Lin-MC3-DMA DOPE Cholesterol DMG-PEG 2000(35%:16%:46.5:2.5% for LNP4)
Cargo	Luc mRNA; SpCas9 mRNA, sgRNA (Rosa26) or sgRNA #1 and/or #23 *DMD*	Luc mRNA;SpCas9 mRNA, sgRNAs (#1 and/or #23 *DMD*)	Luc mRNA, Cre mRNA mOVA (model antigen for mRNA vaccination), mTrp2 mRNA (melanoma antigen for therapeutic vaccination)	Cre mRNA; ABE8.8 mRNA sgRNA (gRNA_A36G).	CRE mRNA; Cas9 mRNA, sgRNA
Amount of encapsulated RNA	10ug of Cas9 mRNA; 5-10ug sgRNA (IM); 1mg/Kg-10mg/Kg total RNA (limb perfusion)	2,4 mg/Kg(IM); 5mg/Kg (IV) total RNA of Luc mRNA or Cas9 mRNA:#sgRNA1:#sgRNA23 at a 2:1:1 ration)	0.3mg/Kg (IM) of Luc mRNA; 0.5mg/Kg (IM) of Cre mRNA;	1.5mg/Kg total RNA (Cre mRNA or ABE8.8 mRNA sgRNA_A36G).	0.625 µg of total RNA (in utero injection)
Route of Administration	Intramuscular; IV limb perfusion	Intramuscular; IV tail vein	Intramuscular, Intradermal, Subcutaneous	IV. Fetal (vitelline vein), Neonatal (temporal vein), Adult (tail vein)	In utero intrahepatic; adult IV
Main Outcome	Stable exon skipping for 1 year (qRT-PCR); restored dystrophin protein (Western blot, inmunofluorescence)	Efficient satellite cell editing; genome editing maintained after muscle injury	High muscle selectivity (iso-A11B5C1); potent cellular immune and antitumor response	Improved survival in mice treated with LNP-ABE at fetal stage.	Targets fetal skeletal muscle, heart and diaphragm.Very low editing efficiency when using Cas9 mRNA instead of Cre mRNA.
Tissue/cell editing	Broad skeletal muscle targeting	Skeletal muscle; Pax7+ satellite cells.	High recombination in muscle; undetectable in liver/spleen.	30% liver (fetal); 7% liver (neonatal); 2-5% cortex (fetal); 1-3% (neonatal)Cortex and spinal cord (endothelial cells; microglia and neurons); Targeted fetal spinal cord microglia (~20%) and neurons (~4%)	Cre mRNA: Td-Tomato expression in heart (5-10%); diaphragm and skeletal muscle (including Pax7+ cells); ~51% diaphragm myofibers Td-Tomato positive post-birth; Cas9 editing in < 0.2% heart.
Off-target analysis	Circle-seq In vitro	NA	NA	NGS	NA
Toxicity Observed	Transient blood cytokines elevation (returned to baseline within 1 week); no significant LNP-mediated liver injury	Transient blood cytokine elevation (returned to baseline within 1 week)	Acute inflammatory cytokine profile in the blood 4 h post IM administration.	>80% survival to P5 across all treatment groups	96.3% fetal survival; 100% survival of the injected mothers; no td-Tomato positive cells in the mother, minimal impact on cytokines in liver