

### GenLNP-A01-eSpCas9 mRNA(m1Ψ)-TRAC sgRNA

GenLNP-A01-eSpCas9 mRNA(m1Ψ)-TRAC sgRNA 是一种加载了增强型特异性 Cas9(eSpCas9)mRNA(m1Ψ) 和 TRAC sgRNA 的脂质纳米颗粒 (LNP) 制剂, 该制剂采用通用 LNP 配方, 主要使用 ALC0315 作为可离子化脂质成分。所加载的 eSpCas9 mRNA (m1Ψ) 经过优化, 其全序列中的所有尿苷 (U) 均被 100% 替换为 N1-甲基假尿苷 (N1-methyl-pseudoUridine, m1Ψ)。TRAC sgRNA 为经 HPLC 纯化的 SafeEdit sgRNA, 具有序列修饰, 靶向 TCRα 基因常链第一个外显子 (TRAC)。通过利用 TCR 基因的内源性转录调控, TRAC sgRNA 可增强 CAR-T 细胞的效能和持久性。在此 LNP 制剂中, eSpCas9 mRNA 与 TRAC sgRNA 按摩尔比 1:10 加载。

该 LNP 制剂是用于体外和体内实验模型的理想对照, 适用于验证 ALC0315 LNP 配方是否适合作为 CRISPR 基因敲除产品的潜在递送平台。

名称	货号	规格
GenLNP-A01-eSpCas9 mRNA(m1Ψ)-TRAC sgRNA	RP-A00019	0.05 mg

**浓度:** 0.15mg/mL

**储存溶剂:** Tris-HCl/Sucrose

**mRNA 全长:** 4471 nt

**mRNA 分子量:** 1457890 Da

**储存条件:** 长期储存请置于 -80°C, 避免冻融, 仅在使用前解冻一次。

#### 细胞实验方法:

实验方法: 将 2.4 μg RNA/LNP 制剂与 HEK293T 细胞共同孵育, 分别在第 3 天和第 5 天裂解细胞, 提取样本进行 PCR 和 Sanger 测序。基因编辑效率数据通过 ICE 分析工具和金斯瑞数据分析软件进行分析。

#### eSpCas9 mRNA ORF 序列:

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ATGCCAAAGAAGAAGCGGAAGGTCGGTATCCACGGAGTCCCAGCAGCCGACAAGAAGTACAGCATCGGCC
TGGACATCGGCACCAACTCTGTGGGCTGGGCCGTGATCACCGACGAGTACAAGGTGCCAGCAAGAAATT
CAAGGTGCTGGGCAACACCGACCGGCACAGCATCAAGAAGAACCTGATCGGAGCCCTGCTGTTTCGACAGC
GGCGAAACAGCCGAGGCCACCCGGCTGAAGAGAACCGCCAGAAGAAGATACACCAGACGGAAGAACCGG
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GTGGCCTACCACGAGAAGTACCCACCATCTACCACCTGAGAAAGAACTGGTGGACAGCACCGACAAGG
CCGACCTGCGGCTGATCTATCTGGCCCTGGCCACATGATCAAGTTCGGGGGCCACTTCCCTGATCGAGGG
CGACCTGAACCCCGACAACAGCGACGTGGACAAGCTGTTTCATCCAGCTGGTGCAGACCTACAACCAGCTG
TTCGAGGAAAACCCCATCAACGCCAGCGGCGTGGACGCCAAGGCCATCCTGTCTGCCAGACTGAGCAAGA
GCAGACGGCTGGAAAATCTGATCGCCAGCTGCCCGGCGAGAAGAAGAATGGCCTGTTTCGGAACCTGAT
TGCCCTGAGCCTGGGCTGACCCCAACTTCAAGAGCAACTTCGACCTGGCCGAGGATGCCAACTGCAG
CTGAGCAAGGACACCTACGACGACGACCTGGACAACCTGCTGGCCAGATCGGCGACCAGTACGCCGACC
  
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TGTTTCTGGCCGCCAAGAACCTGTCCGACGCCATCCTGCTGAGCGACATCCTGAGAGTGAACACCGAGATC  
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AAGCTCTCGTGCGGCAGCAGCTGCCTGAGAAGTACAAAGAGATTTTCTTCGACCAGAGCAAGAACGGCTAC  
GCCGGCTACATTGACGGCGGAGCCAGCCAGGAAGAGTTCTACAAGTTCATCAAGCCCATCCTGGAAAAGAT  
GGACGGCACCAGGAACTGCTCGTGAAGCTGAACAGAGAGGACCTGCTGCGGAAGCAGCGGACCTTCGA  
CAACGGCAGCATCCCCACCAGATCCACCTGGGAGAGCTGCACGCCATTCTGCGGGCGGCAGGAAGATTTT  
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CTGACCAAAGTGAAATACGTGACCGAGGGAATGAGAAAGCCCGCCTTCTGAGCGGCGAGCAGAAAAAGG  
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CTGTTTCGACGACAAAAGTGATGAAGCAGCTGAAGCGGCGGAGATACACCGGCTGGGGCAGGCTGAGCCGG  
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CATCGTGATCGAAATGGCCAGAGAGAACCAGACCACCCAGAAGGGACAGAAGAACAGCCGCGAGAGAATG  
AAGCGGATCGAAGAGGGCATCAAAGAGCTGGGCAGCCAGATCCTGAAAGAACACCCCGTGGAAAACACCC  
AGCTGCAGAACGAGAAGCTGTACCTGTACTACCTGCAGAATGGGCGGGATATGTACGTGGACCAGGAACT  
GGACATCAACCGGCTGTCCGACTACGATGTGGACCATATCGTGCCTCAGAGCTTTCTGGCCGACGACTCCA  
TCGACAACAAGGTGCTGACCAGAAGCGACAAGAACCAGGGGCAAGAGCGACAACGTGCCCTCCGAAGAGGT  
CGTGAAGAAGATGAAGAACTACTGGCGGCAGCTGCTGAACGCCAAGCTGATTACCCAGAGAAAGTTCGACA  
ATCTGACCAAGGCCGAGAGAGGGCGGCTGAGCGAACTGGATAAGGCCGGCTTCATCAAGAGACAGCTGGT  
GGAAACCCGGCAGATCACAAGCACGTGGCACAGATCCTGGACTCCCGGATGAACACTAAGTACGACGAG  
AATGACAAGCTGATCCGGGAAGTGAAAGTGATCACCTGAAGTCCAAGCTGGTGTCCGATTTCCGGAAGGA  
TTTCCAGTTTTACAAAGTGC GCGAGATCAACA ACTACCACCGCCACGACGCCTACCTGAACGCCGTG  
TGGAACCCGCCCTGATCAAAAAGTACCCTGCGCTGGAAAGCGAGTTCGTGTACGGCGACTACAAGGTGTA  
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AGCAACATCATGAACTTTTTCAAGACCGAGATTACCCTGGCCAACGGCGAGATCCGGAAGGCGCCTCTGAT  
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GCTGAGCATGCCCCAAGTGAATATCGTGAAAAAGACCGAGGTGCAGACAGGCGGCTTCAGCAAAGAGTCT  
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TCGAGCTGAAAACGGCCGGAAGAGAATGCTGGCCTCTGCCGGCGAACTGCAGAAGGGAAACGAACTGG  
CCCTGCCCTCCAAATATGTGAACTTCTGTACCTGGCCAGCCACTATGAGAAGCTGAAGGGCTCCCCGAG  
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GGGATAAGCCCATCAGAGAGCAGGCCGAGAATATCATCCACCTGTTTACCCTGACCAATCTGGGAGCCCCT  
GCCGCCTTCAAGTACTTTGACACCACCATCGACCGGAAGAGGTACACCAGCACCAAAGAGGTGCTGGACG  
CCACCCTGATCCACCAGAGCATCACCGGCCTGTACGAGACACGGATCGACCTGTCTCAGCTGGGAGGCGA  
CAAAAGGCCGCGGCCACGAAAAAGGCCGCCAGGCAAAAAAGAAAAAGGAATTCGGCAGTGGA

**TRAC sgRNA 序列:**

mA\*mG\*mA\*GUCUCUCAGCUGGUACAGUUUUAGAGCUAGAAUAGCAAGUUAAAA  
UAAGGCUAGUCCGUUAUCAACUUGAAAAAGUGGCACCGAGUCGGUGCU\*mU\*mU\*mU

(m = 2'O-Methyl RNA; \* = PS 修饰的磷酸二酯键)