TALENs: programmable gene editing proteins

Lipoprotein(a) and cardiovascular disease

Designed TALENs targeting LPA

LUNAR® LNP Technology

TALEN mRNA

In vitro screening of TALEN mRNAs
Efficacy in Primary Human Hepatocytes

Minimal Off-Target Activity

TALENs reduce Lp(a) in transgenic mouse model

TALENs induce gene editing in transgenic mouse model

REFERENCES

SUMMARY

• Achieved proof-of-concept for TALEN-mediated Apo(a) reduction in vitro using TALEN mRNAs
• Demonstrated in vivo efficacy by LNP delivery of TALEN mRNA that led to Lp(a) reduction in a transgenic mouse model
• TALEN-mediated gene editing was dose responsive
• TALEN mRNA-LNP formulations were well-tolerated in vivo
• No detectable off-target activity in vivo

TALENs delivered as mRNA cargo in LNPs cause LPA gene editing and Lp(a) reduction in vivo.

Demonstrated

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REFERENCES


TALEN-Mediated Gene Editing of LPA Causes Gene Disruption and Plasma Lipoprotein(a) Reduction in Transgenic Mice.

Daniel A. Garcia, Alipal Pierre, Linda Quirino, Grishma Acharya, Ashwarya Vasudevan, Yihua Pei, Michael Endow, Kristen Kuski, Michael Broshahai, Maria Chumipdz, Adrian Bukunov, Sumantie Pakur, Paul Chovasala, Stefan A. Bratems, Ramion Diaz-Trelles
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