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Summary

INTRODUCING MINI INTRON1 IN SPINK1 GENE ELEVATED SPINK1 EXPRESSION AND IMPROVED GENE THERAPY FOR PANCREATITIS IN MICE

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Pancreas

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Addition of a mini intron1 sequence to AAVpan-mediated hSPINK1 gene therapy enhances expression 30-fold and protects against severe acute pancreatitis in mice while maintaining safety.

KEY POINTS

- AAVpan-hSPINK1-mini intron1 achieved a 30-fold increase in hSPINK1 mRNA levels, 7-fold elevation in protein expression, and 1.5-fold improvement in trypsin-inhibition activity compared to AAVpan-hSPINK1 without the mini intron.
- The mini intron1 sequence stabilized hSPINK1 mRNA, with levels remaining stable after actinomycin D treatment while mRNA in the standard construct continuously decayed.
- AAVpan-hSPINK1-mini intron1 attenuated severe acute pancreatitis induced by high-dose caerulein (250 μg/kg/h), whereas the standard AAVpan-hSPINK1 construct showed typical pancreatitis phenotypes.
- Hepatic and renal function, pancreatic histology, and serum amylase remained within normal levels in both treatment groups for up to 8 weeks post-injection, demonstrating safety.
- The authors propose this mini intron strategy as a promising approach for optimizing transgene expression in gene therapy for severe pancreatitis and potentially other diseases.

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