HN22C0307

Developing biomedicine to overcome neuropathic pain by GCH1 expression down-regulation

Yonsei University College of Medicine



NEUROSCIENCE	Hit
Product Type	RNAi-based AAV
Indication	Neuropathic pain
Target	GTP cyclohydrolase 1 (GCH1)
MoA(Mechanism of Action)	Knockdown of overexpressed GCH1 using AAV-u-shGCH1 - Decreased BH4 level, GCH1 expression, inflammatory responses relieving pain
Competitiveness	 First-in-Class Never developed biomedicines using RNAi-based therapy that downregulates BH4 levels and inflammatory responses in neuropathic pain Already holding a patent for GCH1 AAV therapy, which is a rat-specific and simultaneously expressed GFP Able to induce long-term effects even with a single minimum dose injection Its mechanism is distinct from KLS-2031 in terms of mechanism of action
Development Stage	Hit
Route of Administration	Sciatic nerve, spinal cord, intrathecal

