eTable 1: Molecular therapies for genetic disorders in neurology.

Technique/mechanism	Mechanism description	Examples of drugs	Use in clinical neurology
1) Gene replacement therapy	Viral-mediated intracellular delivery of exogenous cDNA encoding for a functional copy of the defective gene	Onasemnogene abeparvovec for SMA	Yes
2) RNA interference	Silencing of disease genes by single-stranded (ASO) or double-stranded (siRNAs) short nucleic acid sequences	Nusinersen for SMA	Yes
3) Small molecules	Organic compounds with low molecular weight that target RNA mechanisms, such as transcription, splicing, and translation.	Risdiplam for SMA	Yes
4) Genome editing by CRISPR-Cas9	Genome editing by Clustered Regularly Interspaced Short Palindromic Repeats (CRISPR) associated Cas9 system derived from the bacterial immune system and used to repair or knock out mutant genes.	NTLA-2001 for hATTR (phase I clinical trial)[1]	No

ASO: antisense oligonucleotide; cDNA: complementary DNA; CRISPR-Cas9: Clustered Regularly Interspaced Short Palindromic Repeats (CRISPR) associated Cas9 system; hATTR: Hereditary transthyretin amyloidosis; siRNA: short interfering RNA; SMA: Spinal Muscular atrophy,

Bibliography

1. Gillmore JD, Gane E, Taubel J, et al. CRISPR-Cas9 In Vivo Gene Editing for Transthyretin Amyloidosis. N Engl J Med. 2021;385:493–502.