CLAIMS

1. Use of a composition comprising a Clustered Regularly Interspaced Short Palindromic Repeats (CRISPR)-CRISPR associated (Cas) (CRISPR-Cas) system in the manufacture of a medicament for the treatment of a brain or neurological disease or disorder, in which the composition comprises one or more viral vectors encoding a CRISPR-Cas system, in which the CRISPR-Cas system comprises a Cas9 comprising at least one nuclear localization sequence, a guide sequence that hybridizes to a target sequence expressed in a brain or neuronal cell and associated with a gene involved in the brain or neurological disease or disorder, a tracr mate sequence and a tracr sequence.

2. Use according to Claim 1, in which upon the use expression of a gene product is altered by the CRISPR-Cas system in the brain or neuronal cell, thereby producing a phenotypic change in the eukaryotic organism.

3. Use according to Claim 1 or 2, in which the target sequence is associated with a gene involved in a brain or neurological disease or disorder.

4. Use according to Claim 1, 2 or 3, in which the CRISPR-Cas system corrects one or more deficient genotypes in the brain or neuronal cell.

5. Use according to any preceding claim, in which the one or more viral vectors comprise:

(a) a first regulatory element operably linked to a polynucleotide sequence encoding the guide sequence, the tracr mate sequence, and the tracr sequence, and

(b) a second regulatory element operably linked to a polynucleotide sequence encoding the Cas9 comprising at least one nuclear localization sequence,

in which components (a) and (b) are located on same or different vectors.

6. Use according to any of Claims 1 to 4, in which the one or more viral vectors comprise:

(a) a polynucleotide sequence comprising, form 5’ to 3’, the guide sequence, the tracr mate sequence, and the tracr sequence, and

(b) a polynucleotide sequence encoding the Cas9 and at least one nuclear localization sequence,

in which the tracr mate sequence hybridizes to the tracr sequence, in which the guide sequence directs sequence-specific binding of a CRISPR complex comprising the Cas9 to the target sequence, and in which the polynucleotide sequence encoding a Cas9 is DNA or RNA.

7. Use according to any one of Claims 1 to 4, in which the one or more viral vectors comprise:

(a) a polynucleotide sequence comprising the guide sequence and the tracr mate sequence,

(b) a polynucleotide sequence encoding the Cas9 and at least one nuclear localization sequence, and

(c) a polynucleotide sequence comprising the tracr sequence,

in which the tracr mate sequence hybridizes to the tracr sequence, in which the guide sequence directs sequence-specific binding of a CRISPR complex comprising the Cas9 to the target sequence, and in which the polynucleotide sequence encoding a Cas9 is DNA or RNA.

8. Use according to any preceding claim, in which upon the use expression of two or more neuronal gene products is altered.

9. Use according to any preceding claim, in which the guide sequence is fused to the tracr mate sequence and the tracr sequence.

10. Use according to any preceding claim, in which the polynucleotide sequence encoding the Cas9 is codon optimized for expression in the eukaryotic organism.

11. Use according to any preceding claim, in which the eukaryotic organism is a mammal.

12. Use according to any preceding claim, in which the Cas9 is *S. pyogenes* Cas9.

13. Use according to any one preceding claim, in which the Cas9 is *S. aureus* Cas9.

14. Use according to any preceding claim, in which the one or more viral vectors is an adeno-associated virus (AAV) or lentiviral vector.

15. Use according to Claim 14, in which the adeno-associated virus (AAV) is AAV1, AAV2, AAV4, AAV5, AAV8, AAV9, or a combination thereof.

16. Use according to Claim 5, 6, or 7, in which (a) and (b) are located on the same viral vector.

17. Use according to Claim 16, in which the viral vector is an AAV vector and the Cas9 is *S. aureus* Cas9.

18. Use according to any preceding claim, in which the viral vectors are for delivery via stereotactic injection.

19. Use according to any preceding claim, in which the polynucleotide sequence encoding the Cas9 is operably linked to a brain-specific promoter or a neuron-specific promoter.

20. Use according to any preceding claim, in which the polynucleotide sequence encoding the Cas9 is operably linked to a promoter selected from the group consisting of CamkII, Parvalbumin, vGAT, DR1, DR2, GFAP, and Arc.