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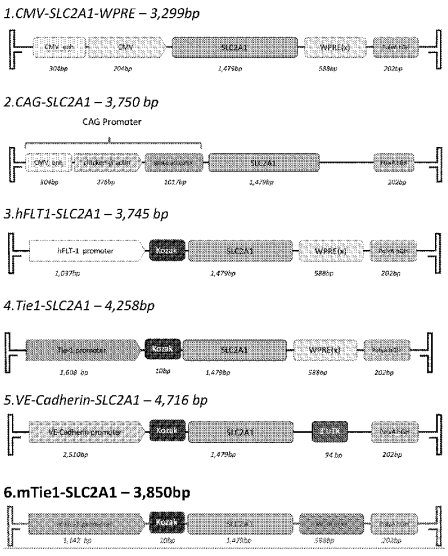
(54) Title: ADENO-ASSOCIATED VIRAL VECTOR FOR GLUT1 EXPRESSION AND USES THEREOF

FIG. 1



(57) Abstract: Provided herein is a gene therapy for GLUT1 Deficiency Syndrome and related disorders using a recombinant adeno-associated virus (rAAV) virion as a vector to express an GLUT1 protein or functional variant thereof. The rAAV virion may use an endothelial-specific promoter, *e.g.*, a FLT-1 or Tie-1 promoter. The capsid may be an AAV6, AAV8, AAV9, AAVrh.74, or AAVrh.10 capsid or a functional variant thereof. Other promoters or capsids may be used. Further provided are methods of treatment, such as by intracerebrally and/or intravenously of the rAAV virion, and other compositions and methods.

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— *as to applicant's entitlement to apply for and be granted a patent (Rule 4.17(ii))*
— *as to the applicant's entitlement to claim the priority of the earlier application (Rule 4.17(iii))*

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