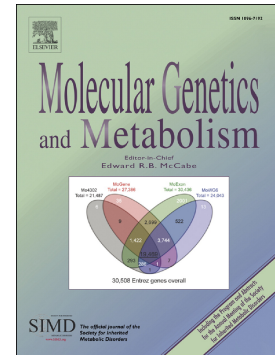


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## Treatment of brain disease in the mucopolysaccharidoses

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AAV: adeno-associated viruses, AE: adverse event, BBB: blood-brain barrier, CLN2: ceroid lipofuscinosis neuronal type 2, CNS: central nervous system, CSF: cerebrospinal fluid, DQ: developmental quotient, ERT: enzyme replacement therapy, g7: 7-aminoacid glycopeptide, GAG: glycosaminoglycans, HSCT: hematopoietic stem cell transplantation, ICV: intracerebroventricular, IDDD: IT drug delivery device, IQ: intelligence quotient, IT: intrathecal, LAMP-1: lysosomal-associated membrane-1, MPS: mucopolysaccharidosis, MRI: magnetic resonance imaging, SCC: spinal cord compression, SGSH: N-sulfoglucosamine sulfohydrolase, SRT: substrate reduction therapy, SUMF: sulfatase modifying factor

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