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# OVERCOMING ADENO-ASSOCIATED VIRUS GENE THERAPY LIMITATIONS IN GENETIC NEUROMUSCULAR DISEASES

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Neuromuscular diseases represents a major hurdle for patients, families and the society in its entirety. Rare, genetic neuromuscular diseases constitute a bigger challenge given the absence of knowledge on the physiopatological mechanisms and of therapeutic options. In the last years, adeno associated virus (AAV) vector-based gene therapy became a principal actor in the development of therapies for monogenic diseases. Successful human trials of gene transfer in the liver for hemophilia A and B, in the eye for congenital blindness and in the nervous system for spinal muscular atrophy have unveiled the therapeutic potential of this viral vector platform. However, one of the main limitation of AAV gene therapy is the high dose of vector needed to rescue neuromuscular diseases. Doses used in the clinic for these disorders are hardly produced and are likely to induce unwanted secondary effects. Another constraint in the use of AAV vector is their limited size of transgene encapsidation. This is of particular relevance in genetic neuromuscular diseases that frequently involve large transgenes. Here, using glycogen storage disorders as model diseases, we developed some technological tools to overcome the current limitations of AAV gene therapy applied to neuromuscular diseases.