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(54) **Title:** COMPOSITIONS USEFUL FOR TREATING SPINAL AND BULBAR MUSCULAR ATROPHY (SBMA)

(57) **Abstract:** Compositions useful for treatment of Spinal and Bulbar Muscular Atrophy (SBMA) comprising administration of a recombinant adeno-associated virus (rAAV) vector having an AAV capsid and a vector genome comprising a sequence encoding at least one hairpin forming miRNA that comprises a targeting sequence which binds a target site on the mRNA of human androgen receptor, wherein the miRNA inhibits expression of human androgen receptor, is provided. Also provided are compositions containing a rAAV vector and methods of treating SBMA in patient comprising administration of a rAAV vector.

WO 2022/221276 A1