

# Download Muscular System Advanced Quick Study Academic

Spinal muscular atrophy (SMA) is a rare neuromuscular disorder characterised by loss of lower motor neurons and progressive muscle wasting, often leading to early death.. The disorder is caused by a genetic defect in the SMN1 gene, which encodes SMN, a protein widely expressed in all eukaryotic cells (that is, cells with nuclei, including human cells) and necessary for survival of motor neurons.

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A study published in Science documents how scientists for the first time used gene editing to halt the progression of Duchenne muscular dystrophy (DMD) in dogs. It is seen as a major step toward a clinical trial. CRISPR gene editing technology restored muscle function in dogs to near-normal levels in the heart, diaphragm and other muscles.

Dr. Tsai, Assistant Professor of Neurology & Neurotherapeutics, cares for children with autism and cerebellar disorders as part of the Center for Autism & Developmental Disabilities, a joint program between UT Southwestern and Children's Medical Center Dallas. He collaborated on the study with Harvard Medical School, the University of Toronto, and the Hospital for Sick Kids in Toronto.