Gene therapy leads to long-term benefits in dog model of devastating childhood disease

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Researchers who previously showed that a gene therapy treatment could save the lives of dogs with a deadly disease called myotubular myopathy—a type of muscular dystrophy that affects the skeletal muscles—have found that the therapy is long-lasting. The results support a clinical trial in patients.

Myotubular myopathy is estimated to affect 1 in 50,000 male births, and boys born with the condition often die in the first year of life.

In this latest study, investigators found that replacing the MTM1 gene, which is mutated in patients, leads to sustained preservation of muscle strength and neurologic function in dogs over 4 years.

"This regenerative technology, termed AAV gene transfer, provided long-lasting benefit to the entire musculature of affected dogs that would have otherwise perished, extending a healthy lifespan for more than 4 years," said Dr. Martin Childers, senior author of the *Muscle & Nerve* study and a UW Medicine researcher in Seattle.

More information: Matthew Elverman et al, Long-term effects of systemic gene therapy in a canine model of myotubular myopathy, *Muscle & Nerve* (2017). DOI: 10.1002/mus.25658

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