

Degenerative Myelopathy

What is Degenerative Myelopathy?

- ◆ Degenerative myelopathy (DM) is a devastating neurodegenerative disease that affects multiple breeds of dog.
- ◆ DM is an adult-onset disease that manifests at the later stages of life.
- ◆ It is characterized by progressive weakness and inability to control hindlimbs, ultimately leading to involvement of forelimbs and complete paralysis.

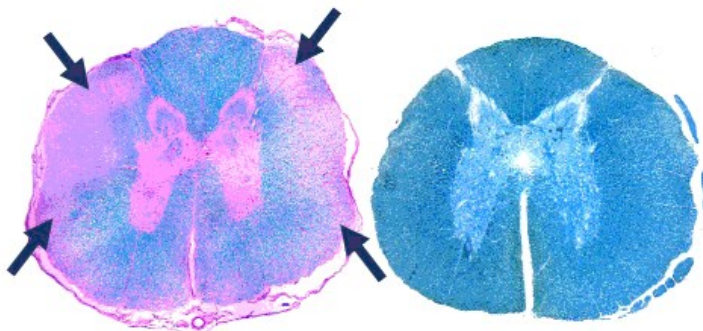


Why study it?

- ◆ With no current treatments available, euthanasia is the only option available for DM-affected dogs.
- ◆ Recent studies have identified mutation in the Superoxide dismutase 1 (SOD1) gene to be a high risk factor associated with canine DM. In humans, mutations in the same SOD1 gene cause Amyotrophic Lateral Sclerosis (ALS) or Lou Gehrig's disease, a neurodegenerative disorder very similar to canine DM.
- ◆ Therapeutic approaches to reduce the expression of mutant SOD1 in DM-affected dogs may improve survival and preserve neurologic function.

Outcome

A research study ([2165](#)) funded by the AKC Canine Health Foundation and the American Boxer Charitable Foundation has led to a recent [report](#) showing a promising biomarker for dogs with nervous system diseases. Similar biomarkers are being studied in ALS patients and findings from this study may also impact human treatments.



University of Missouri

The left image shows a section of a spinal cord from a dog who has died of DM; the degeneration is seen as a loss of the blue color at the edges (arrows).

The right image shows a normal spinal cord.



CHF-Funded Study:

02210: Gene Therapy for Canine Degenerative Myelopathy

Principal Investigator: Dr. Brian K Kaspar, PhD; The Research Institute at Nationwide Children's Hospital

Total Grant Amount: \$50,000

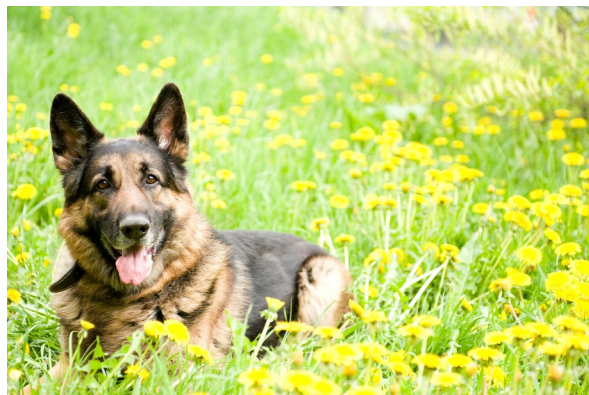
Grant Period: 1/1/2016 – 12/31/2018

What is the focus of this study?

- ◆ In this study, a viral-based gene therapy approach to treat DM will be evaluated, utilizing Adeno-associated Virus 9 (AAV9) mediated delivery of shRNA to reduce the mutant SOD1 in DM affected dogs.
- ◆ AAV9 is a safe, well tolerated and widely used vector for gene therapy in animals as well as for humans.

How will this study help dogs?

- ◆ If successful, this one-time treatment with AAV9 SOD1 shRNA will result in improved quality of life, and significantly extend the survival of dogs affected with this previously hopeless disease.



To learn more or make a donation, please visit www.akcchf.org/DM