



Tay-Sachs Sheep HOPE for a Cure

Doug Martin, PhD, Auburn University, received Research Initiative Grants from NTSAD to characterize natural disease progression in the Tay-Sachs sheep model and therapeutic benefits of gene therapy in Tay-Sachs sheep.

For two years, sheep with Tay-Sachs were studied in a controlled research environment to clearly chart the disease course in this newly identified animal model. This study is important because it provides a baseline to measure therapeutic interventions such as gene therapy. Here is a summary of the results:

- 1.** The lifespan of an untreated Tay-Sachs sheep is approximately 9 months with disease onset around 2.5 months.
- 2.** Most disease signs in Tay-Sachs sheep relate to gait abnormalities, such as hind limb weakness or ataxia, that worsen with age.
- 3.** All Tay-Sachs sheep have clouding of the eye's cornea but none have the 'cherry-red spot' found in children which is likely due to differences in eye structure.
- 4.** No startle or hearing abnormalities were observed.

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