

# Interview on gene therapy with board member Nerry Kamstrup

Once a year the World Small Animal Veterinary Association WSAVA hosts a congress for companion animal veterinarians from across the globe, and this year it was held in Copenhagen. The Danish Society of Veterinary Ophthalmology, DSVO, saw the opportunity to have the Nordic Eye Meeting hosted in Denmark in connection to the congress.

Panion's board member Nerry Kamstrup, who is a veterinary practitioner and ophthalmologist in her daily life, participated and she explains: "I had the pleasure to receive the latest information of what my colleagues are doing around the world. The congress was great, but sometimes the most fascinating knowledge comes during a lunchbreak as it did for me this year. Having lunch with an old colleague, Professor Ron Ofri from the Koret School of Veterinary Medicine at the Hebrew University of Jerusalem, gave me new insight of the applied gene therapy where animal science overlaps with human developmental medicines."

Professor Ofri had investigated Awassi sheep flocks where some lambs were born with congenital visual impairments that differed from other known forms of blindness in sheep. The pedigree analysis suggested an autosomal recessive mode of inheritance, and behavioral studies showed that the day vision (but not the night vision) of the sheep was impaired – a so-called Day-time blindness. The evaluation of affected eye retinas reflected cone dysfunction rather than cone photoreceptor loss.

This resembled an inherited condition in humans called Acromatopsia associated with loss of visual acuity,

extreme light sensitivity, resulting in daytime blindness, and reduced or complete loss of color discrimination. There is no specific treatment for this genetic disease.

In a study in sheep, by using a gene therapy method to deliver an AAV-vector carrying the correct copy of the mutated gene into the eyes, it was shown that the vision could be restored, which was measured by the sheep's ability to navigate in a maze. This demonstrated the proof-of-concept for the treatment and the next step was to use this in the technology for treatment of the human form of Achromatopsia, Day-time blindness.

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Using naturally occurring disease in sheep (and in dogs) as models of the disease in humans, is a great way of coupling the animal and the human scientific developments for the benefit of both fields. There are now programs that investigate how these findings can be used in human Achromatopia, and there are clinical studies running to evaluate the safety and efficacy.

“These are very exciting news as the work and progress of gene therapy advances with the hope that a single injection could provide long lasting recovery for animals and humans.” says Nerry Kamstrup.

She sees a clear parallel to Panion’s work in epilepsy, where discoveries initially intended for the human field in the coming years will be explored for the treatment of dogs with epilepsy as well.



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**Developing animal health** – In Panion, we want to improve the quality of life for animals suffering from chronic diseases. We are convinced that gene therapy has promising prospects. Our aim is to develop and commercialize a gene therapy treatment for dogs with drug refractory epilepsy, based on CombiGene AB’s technology and platform. Panion Animal Health AB is listed at AktieTorget.

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