

Joint DANDRITE-Biomedicine/Neuroscience Seminar

with visitor

Dr Rikke Hahn Kofoed



**Friday 20th December 2019 at 11:00, Auditorium 1170-347,
Ole Worms Allé 3 8000 Aarhus C**

Non-invasive delivery of gene therapy to the CNS using MRI-guided focused ultrasound and microbubbles

Gene therapy holds the promise to provide long-lasting clinical benefits for neurodegenerative disorders following a single administration. However, adeno-associated viral vectors (AAVs), commonly used for gene therapy, do not easily bypass the blood-brain barrier (BBB) and efficient AAVs delivery to the CNS requires either very high doses or invasive surgery. Pioneer work at Sunnybrook has established that MRI-guided focused ultrasound (MRIGFUS) in combination with microbubbles can safely, transiently and non-invasively increase the permeability of the BBB in targeted areas of the brain and spinal cord. For gene therapy, MRIGFUS offers the advantage to deliver AAVs, administered intravenously at a relatively low dose, to specific areas of the CNS. In this talk I will present data from our investigations of novel AAV capsids and promoters, and demonstrate how MRIGFUS and AAV can be combined to enable both localized, widespread and disease-regulated transgene expression in the CNS.

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