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Drug discovery for cerebellar ataxias: Novel strategies

Cerebellar ataxia is a rare neurological disorder characterized by the inability to control voluntary movements, and can manifest among other as gait ataxia, intention tremor or dysmetria. Genetically, cerebellar ataxias are a heterogeneous group of related disorders, with different modes of inheritance. Episodic ataxias are caused by mutations in ion channels, most notably KV1.1 and CaV2.1. However, to date there is no confirmed treatment for cerebellar ataxias, and response rates for existing drugs are generally low. Recent advances utilizing novel transgenic model systems that closely resemble the human condition have brought about a paradigm change that requires us to revise our strategies for drug discovery and development for cerebellar ataxias. In this talk, I will present the latest experimental findings in basic and translational research, discuss new drug targets and outline novel strategies for drug discovery that focus on restoring synaptic transmission in the CNS.

Biography

Simon Kaja is an experienced neurobiologist with a long-standing research track record in academia and the biotech and pharmaceutical industry. Dr. Kaja currently serves as Associate Director of Preclinical Research at the Vision Research Center at the University of Missouri - Kansas City, School of Medicine. He is the Director of Microscopy of the imaging core facility at the Vision Research Center and also holds an appointment to Assistant Professor of Ophthalmology. The focus of his research program centers around human neurological and neurodegenerative diseases and visual disorders. He obtained his B.Sc. (Hons.) degree in Molecular Biology and Biochemistry from Durham University, UK and holds a Ph.D. degree in Medicine/Neuroscience from Leiden University, The Netherlands. Prior to joining the faculty at the University of Missouri - Kansas City, he has performed postdoctoral work at the University of British Columbia (Vancouver, B.C., Canada) and the University of North Texas Health Science Center (Fort Worth, TX). In addition to his academic work, he has worked and consulted for a number of pharmaceutical companies, incl. Novo Nordisk A/S, Bayer AG, Neuromed Pharmaceuticals Inc., and NeuroSearch A/S. He is CEO and co-founder of K&P Scientific LLC, a scientific consulting company headquartered in Kansas City, MO.

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