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Therapeutics for rescue of Ikbkap deficient dorsal root ganglia neurons: An In-vitro Approach

The Lefcort lab has developed a mouse model that recapitulates the hallmarks of Familial Dysautonomia by conditionally deleting the IKAP protein from neural crest cells, which gives rise to the neurons and glial cells of the peripheral nervous system, via the Wnt1-cre driver. The mouse shows significant reductions in neuronal number in the dorsal root, sympathetic, and parasympathetic ganglia. As such, it provides an excellent model system for determining the function of IKAP in embryonic development and for mimicking the Familial Dysautonomia found in humans. This research projects aims to assess the neuroprotective effects of two different drugs, Metformin and the GSK2606414 PERK inhibitor, on the Ikbkap deficient dorsal root ganglia (DRG) neurons within this mouse model. The ultimate goal of this study is to identify and target new cell signalling pathways and their neuroprotective potential as they relate to Familial Dysautonomia. An absolute cure for the disease is improbable due to the widespread devastation it causes. However, determining a mechanism to rescue neuronal death could lead to valuable therapeutic approaches and new rescue strategies that could eventually be used to increase the quality of life and overall lifespan in the mice and, eventually, in humans.